Q1 2021 Results

Investor presentation

NOVARTIS | Reimagining Medicine

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Chief Executive Officer

Company overview

Summary of Q1 performance

Growth[1,2]: growth drivers continue momentum 1

- Group sales -2%; Ex. PY forward purchasing **+1%**
- IM sales in line vs. PY; Ex. PY forward purchasing **+3%**
- Sandoz sales -13%; Ex. PY forward purchasing **-9%**

Entresto® sacubitril/valsartan | zolgensma® | Cosentyx® (secukinumab) | PROMACTA® (eltrombopag) | Kesimpta® (ofatumumab) | KYMRIAH® (tisagenlecleucel)

Productivity[1,2]: strong underlying core OpInc of IM 2

- Core operating income -8%; Ex. PY forwarding purchasing **-1%**
- IM core operating income -1%; Ex. PY forward purchasing **+6%**
- IM core margin 36.3% (-0.5%pt cc); Ex. PY forward purchasing **+1%pt**

Innovation: confident in our leading pipeline 3

Entresto®	FDA expanded indication in chronic heart failure (LVEF below normal)
LNP023	Ph2b IgAN **met primary endpoint** enabling Ph3 initiation
^{177}Lu-PSMA-617	Ph3 VISION study **met both primary endpoints** (mCRPC)
Tislelizumab	In-licensing closed. Ph3 positive results in esophageal and NSCLC

ESG: progress recognized 4

access to medicine index #2 | SUSTAINALYTICS #1[3]

- Recognized Novartis for systematic approach to access planning
- The **only** company amongst peers with a "**Low Risk**"
- Integration of ESG in US and clinical trial diversity

All growth % in cc IM – Innovative Medicines division 1. Constant currencies (cc), core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 36 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates in this Release refer to same period in prior year 2. Growth excluding prior year COVID-19 related forward purchasing is a non-IFRS measure, an explanation for this measure can be found on page 44 of the Condensed Interim Financial Report 3. Within peer group as defined by Sustainalytics

Key growth drivers and launches continue momentum in Q1

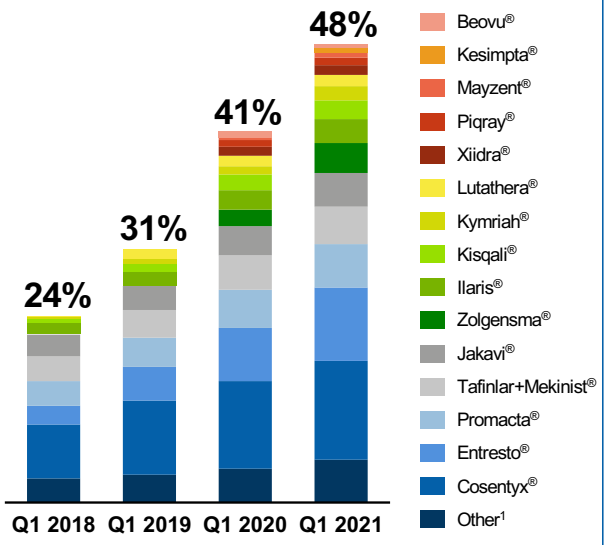
Key growth driver sales momentum[1]

	Q1 Sales USD Million	Growth vs. PY USD Million	Growth vs. PY cc
Entresto® sacubitril/valsartan	789	220	34%
zolgensma®	319	149	81%
Cosentyx® (secukinumab)	1,053	123	11%
PROMACTA® (eltrombopag)	463	60	13%
KYMRIAH® (tisagenlecleucel)	151	58	55%
Kesimpta® (ofatumumab)	50	50	nm
JAKAVI® ruxolitinib	363	45	8%
ILARIS® (canakinumab)	256	43	20%
KISQALI® ribociclib	195	34	19%
Xolair® Omalizumab	335	28	3%
Tafinlar® + Mekinist®	393	27	4%
MAYZENT® (siponimod) tablets	55	25	80%

nm – not meaningful

Driving portfolio rejuvenation

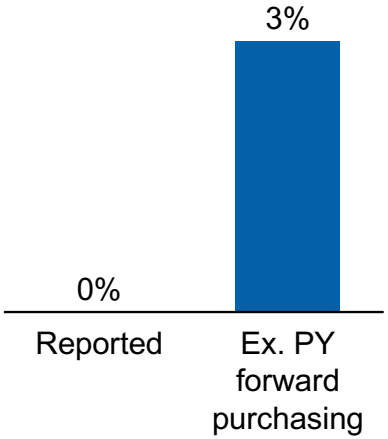
Key growth drivers and launches 48% of IM sales, growing 20% in Q1



1. Includes Xolair®, Aimovig®, Adakveo®, Tabrecta ®, Luxturna® , Enerzair ®, Atectura® and Leqvio®

3% growth ex. PY forward purchasing (reported in-line)[1,2]

Innovative Medicines Q1 net sales growth, % cc

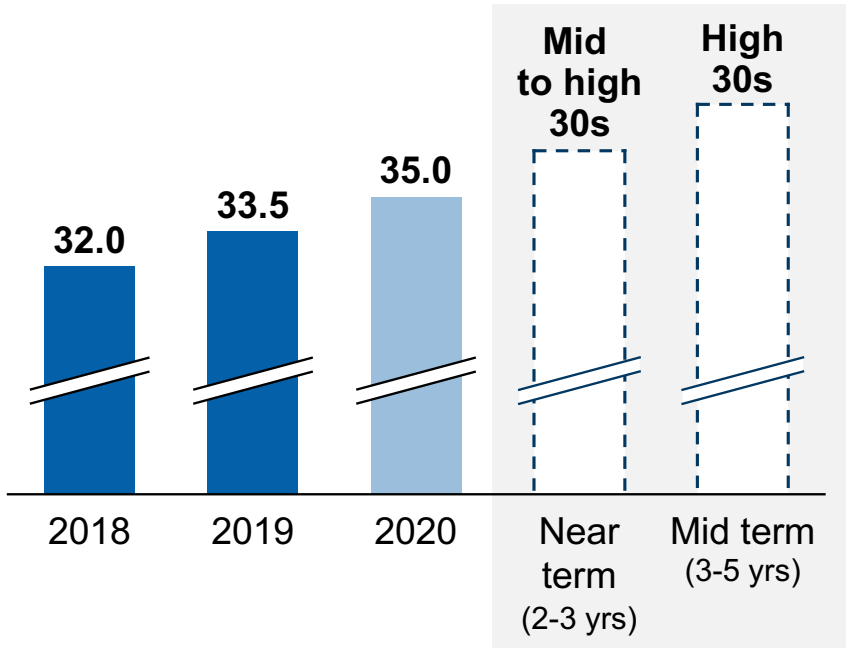


1. Innovative Medicines division. Constant currencies (cc) is a non-IFRS measure. An explanation of non-IFRS measures can be found on page 36 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates in this Release refer to same period in prior year. 2. Growth excluding prior year COVID-19 related forward purchasing is a non-IFRS measure, an explanation for this measure can be found on page 44 of the Condensed Interim Financial Report

Confident to deliver on Innovative Medicines (IM) core margin target; underlying IM Core OpInc +6% ex. PY forward purchasing

Innovative Medicines

Core margin (%)[1]



Q1 IM performance ex. PY forward purchasing

% cc vs. PY

Core OpInc +6%
reported -1%

Core margin +1%pt
reported -0.5%pts
at 36.3%

Key drivers of expansion

- **Sales momentum** of key growth drivers and operational excellence on upcoming launches
- **NTO productivity** program expected to deliver USD 2bn in the mid term
- Evolution in **ways of working**, e.g. CTS integrating data and digital with former NBS, integrating Gene Therapies into Pharma BU

1. Core margin in USD

1. GROWTH | 2. PRODUCTIVITY

Sandoz Q1 performance impacted by price erosion, PY forward purchasing and historically low cough and cold season

Cycling an exceptionally strong PY quarter...

(vs. PY, in cc)

Net total sales
Q1 2020: +11%
Q1 2021: -13%

Net Biopharma sales
Q1 2020: +31%
Q1 2021: +7%

Core operating income
Q1 2020: +53%
Q1 2021: -35%

... impacting both price and volume in Q1

Price erosion	**-10%** Impact on sales
Forward purchasing	**-4%** Impact on sales Cycling Q1 2020 demand surge
Other volume factors	↓ Historically weak cough & cold season ↓ Soft Retail demand ↓ US Oral Solids partnership terminations ↑ Biopharma growth

Business stabilization expected in H2 as pandemic impact eases

Biosimilars	Outperforming in a competitive European market
Retail Europe	Set to benefit from leading market share as market recovers in H2
Launches	2021 launches, primarily in H2

3. INNOVATION

Broad pipeline of novel medicines continued to progress in Q1

Approvals

Entresto®	Expanded heart failure with LVEF below normal (US)
Kesimpta®	EU and JP for rMS
Cosentyx®	EU label update for axial manifestations of PsA

Readouts

- Positive
- Neutral
- Negative

● ^{177}Lu-PSMA-617	Ph3 - mCRPC (VISION)
● Cosentyx®	Ph3 - JIA
● LNP023	Ph2 - IgAN[1] (Ph3 started)
● LNP023	Ph2 - PNH[2] (Ph3 started)
● Entresto®	Ph3 - Post-AMI[3]
● ACZ885	Ph3 - NSCLC 2L

Submissions

JAKAVI® ruxolitinib	EU and JP for acute and chronic GvHD
TABRECTA™ (capmatinib) tablets	EU for NSCLC

Designations

ABL001 asciminib	FDA Breakthrough Therapy designation in CML
BYL719 alpelisib	EU Orphan designation in PROS

All abbreviations on slide 136 1. IgAN Ph2 data to be published at upcoming medical congress 2. Ph2 in PNH anti-C5 treatment naive patients 3. Numerical trends consistently favored Entresto® vs. active comparator but did not meet primary composite endpoint. The safety profile of Entresto® was confirmed

3. INNOVATION

Moving forward a breadth of assets to drive long-term growth

Selected opportunities, **expected 2021 milestones** and additional indications

Lifecycle management

Entresto®	Post-AMI: PARADISE; **topline Ph3 results to be shared at ACC 5/2021**
	HFpEF: **FDA approved Q1 2021**
Cosentyx®	HS: SUNRISE, SUNSHINE **Ph3 readout H2 2021**
	L. Planus, Peds PsO, jPsA/ERA, GCA, lupus nephritis
Kisqali®	aBC: MONALEESA-2 OS **readout H2 2021**
	HR+/HER2- BC (adj) **readout 2022**
Leqvio®	Hyperlipidemia: **CRL response Q2-Q3 2021**
	CVRR-LDLC
Beovu®	DME: **submission H1 2021**
	RVO, diabetic retinopathy

Pharmaceuticals

Iptacopan (LNP023)	IgAN[1], PNH, aHUS: **Ph3 start 2021**
	C3G: **Ph2 readout H1 2021**, iMN
Iscalimab (CFZ533)	Sjögren's, kidney Tx, liver Tx
Ligelizumab (QGE031)	CSU: PEARL 1, 2 **Ph3 readout H2 2021[2]**
	CINDU, food allergy **Ph3 start H2 2021**
Pelacarsen (TQJ230)	CVRR-Lp(a)
Branaplam (LMI070)	HD: **Ph2b start H2 2021**
	SMA

Oncology

Canakinumab (ACZ885)	NSCLC 1L: CANOPY-1 **Ph3 readout H2 2021**
	NSCLC adjuvant
^{177}Lu-PSMA-617	mCRPC 3L: VISION **positive readout; submission H2 2021**
	mCRPC pre-taxane, mHSPC: **Ph3 start H1 2021**
Sabatolimab (MBG453)	HR-MDS: STIMULUS **Ph2 CR readout H2 2021**
	AML
TNO155	Solid tumors, multiple combinations being explored in on-going trials
Tislelizumab (VDT482)	Esophageal cancer and NSCLC: **submission 2021**

'Wild Cards'

ECF843 (Dry eye: **Ph2 readout H2 2021**), LNA043 (Osteoarthritis: **Ph2b start H1 2021**), CSJ117 (Asthma), QBW251 (COPD), LXH254 (BRAF/NRASm melanoma, mRAS/RAF NSCLC), NIS793 (Solid tumors)

ACC – American College of Cardiology 1. IgAN Ph2 data to be published at upcoming medical meeting 2. Q4/2021-Q1/2022 potential COVID-19 impact

3. INNOVATION

Completed in-licensing deal for tislelizumab; ex-China filing for first two indications planned by year end

1st global pivotal study of tislelizumab in 2L NSCLC presented at AACR

- RATIONALE 303 study vs. docetaxel reinforces tislelizumab's competitive profile
- Primary endpoint: mOS ITT 17.2 vs. 11.9 mos; HR=0.64
- Primary endpoint: mOS PD-L1 ≥25% 19.1 mos vs. 11.9 mos; HR=0.52 ($p < 0.0001$[2])
- Safety profile consistent with other tislelizumab mono studies and other PD(L)-1s

Study in 2L ESCC also met its primary endpoint, prolonging OS vs. chemo

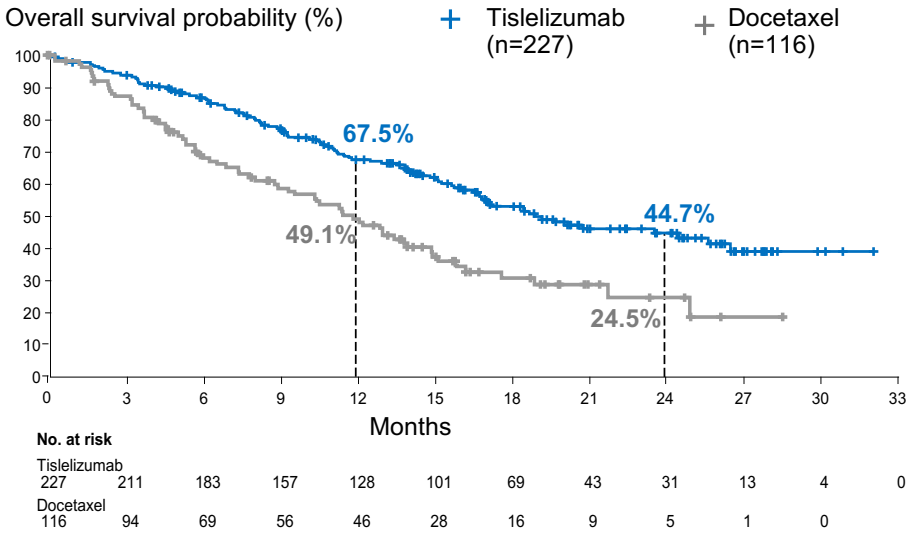
- RATIONALE 302 data to be presented at an upcoming medical congress

1st two ex-China filings in ESCC and NSCLC on track for 2021

- Advancing broad development program: 15 potentially registration enabling studies
- Evaluating and prioritizing potential combination across Novartis portfolio

RATIONALE 303 (AACR 2021)

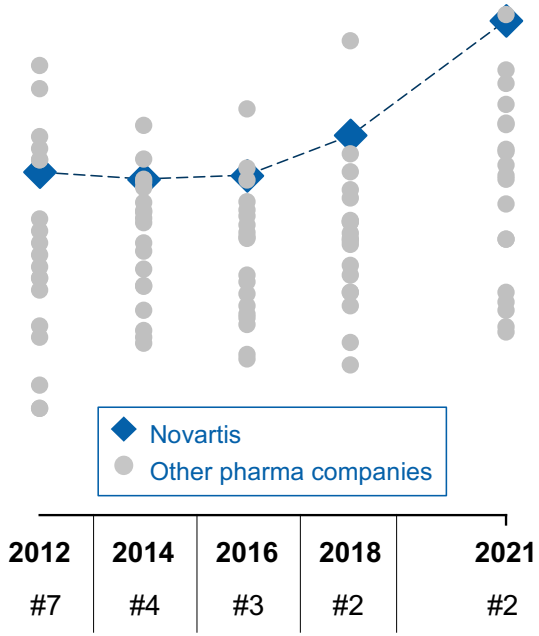
Primary endpoint – overall survival (PD-L1 ≥25%)[1]



mOS – Median overall survival ITT – Intent to treat population Ex-China regions include the US, Canada, Mexico, the EU, UK, Norway, Switzerland, Iceland, Liechtenstein, Russia, and Japan; BeiGene retains the rights to tislelizumab in China and other countries For references, please see slides 48-49

Continued progress on access and global health, recognized with a no.2 ranking in ATMI, no.1 ranking in Sustainalytics[1]

Access To Medicines Index ranking No.2



#1
Product delivery: leader in sustainable equitable pricing strategies

#2
Governance of access: Access Principles, linked to incentives

#3
R&D: comprehensive access plans for late-stage R&D projects

ATMI:
Novartis as **first company** with a systematic approach to access planning (since 2018)

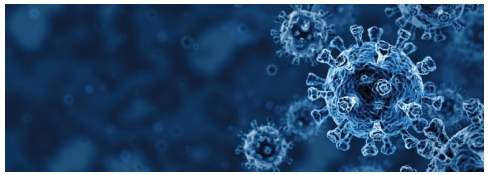
Sustainalytics ranking no.1[1]

The **only** company amongst peers with a "**Low Risk**" rating (improved from "Medium Risk" in 2021)

Sickle cell disease

Bill & Melinda Gates foundation collaboration: addressing disparity in access for sickle cell disease

COVID-19



Manufacturing capacity for COVID-19 vaccine / therapeutic production[2]
Collaboration with Molecular Partners[3]

Bloomberg Gender-Equality

Novartis included again in the 2021 Bloomberg Gender-Equality Index

ATMI – Access to Medicines Index 1. Pharmaceuticals subindustry category & amongst peers with similar market cap 2. Agreement to leverage Novartis manufacturing capacity / capabilities to support production of 1) Pfizer-BioNTech vaccine; 2) CureVac vaccine candidate CVnCoV; 3) API for Roche's Actemra®, being tested for COVID-19 3. To develop, manufacture, commercialize 2 antiviral DARPin® candidates, ensovibep (MP0420) & MP0423

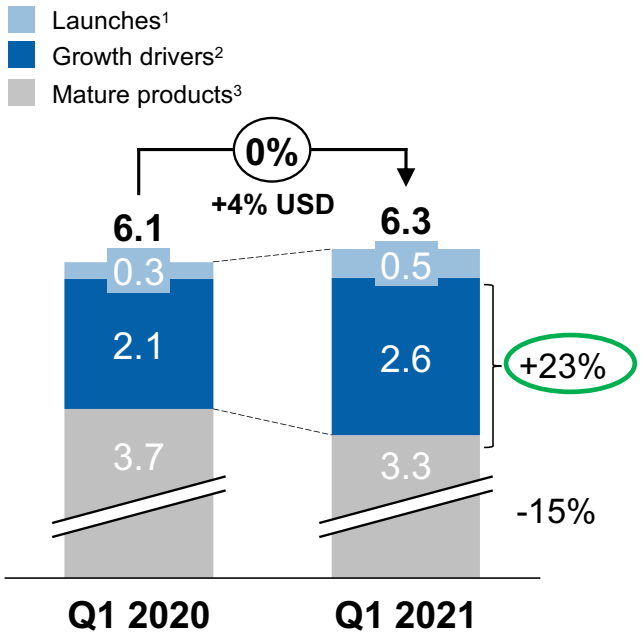
Marie-France Tschudin

President, Novartis Pharmaceuticals

Pharmaceuticals portfolio continues to rejuvenate. Growth drivers and launches almost half of Pharmaceuticals sales, growing 23%

Pharmaceuticals net sales

USD bn, % cc



Growth drivers showing strong momentum vs. prior year

- Cosentyx® and Entresto® contribute USD 1.8bn, growing +19% YoY
- Zolgensma ® grows 81% driven by geographic expansion
- Ilaris® grows 20% driven by Still's disease and Periodic Fever Syndrome[4]
- Xiidra® sales increased 20% YoY

New portfolio laying foundation for future growth

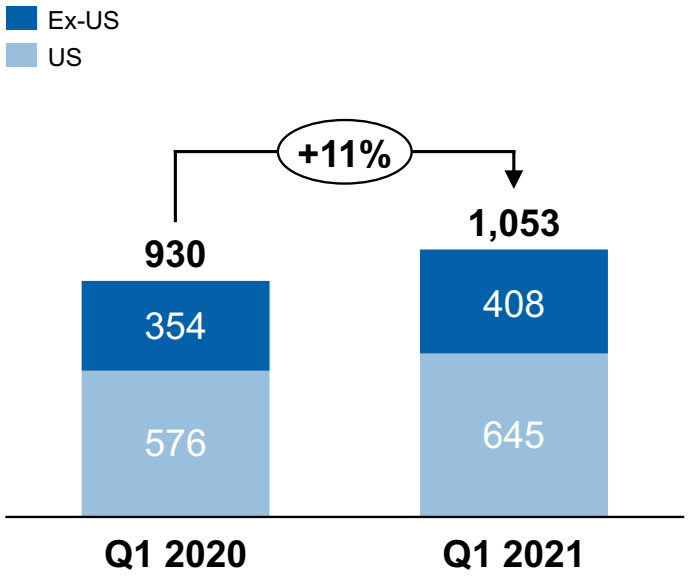
- Growth drivers and launches represent 49% of sales (up from 40% Q1 2020)
- Entresto® expanded label approved in US to include majority of CHF patients
- Kesimpta® approved in EU / JP

All % growth relate to cc unless otherwise stated CHF – Chronic heart failure 1. Zolgensma®, Kesimpta®, Mayzent®, Beovu® , Luxturna®, Leqvio®, Enerzair® and Atectura® 2. Cosentyx®, Entresto®, Xolair® , Ilaris® , Xiidra® and Aimovig®
3. All other brands 4. Adult-onset Still's disease indication launch in US, Periodic Fever Syndrome reimbursement in UK and France

Cosentyx® delivers double digit growth. Momentum expected to continue through 2021

Sales evolution

USD m, % cc



Expecting double-digit FY 2021 growth

- Solid growth despite Q1 access changes and continued COVID-19 impact (visits at 80-90% of pre-COVID-19 baseline[1])
- US lower volume related to access, offset by rebate upside
- Majority of US business first line, with strong access
- Only interleukin inhibitor on NRDL China for PsO and AS

Confident for future growth based on data and LCM

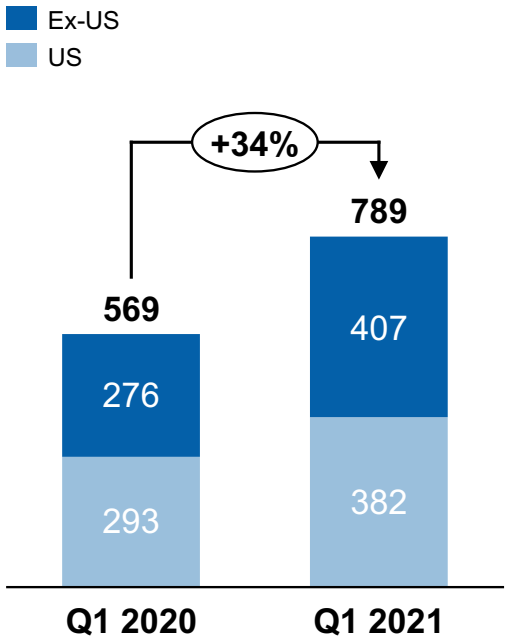
- MAXIMISE data on axial efficacy in PsA included in EU label, further reinforcing profile as complete treatment
- Pediatric indications further reinforce safety profile (PsO US Q2 2021, jPsA & ERA H1 2022)
- Hidradenitis Suppurativa, if approved, adds ~400k addressable patients

NRDL – National Reimbursement Drug List PsO – Psoriasis AS – Ankylosing spondylitis PsA – Psoriatic arthritis jPsA – Juvenile psoriasis arthritis ERA – Enthesitis related rheumatoid arthritis 1. IQVIA Visits Data Dermatology

Entresto® growing 34%. Strong momentum as essential first choice treatment in chronic heart failure

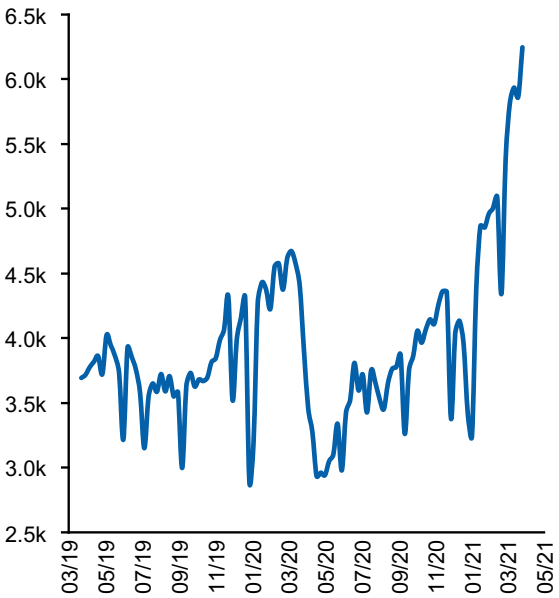
Sales evolution

USD m, % cc



Weekly NBRx[2]

New-to-brand prescriptions



Strong momentum worldwide

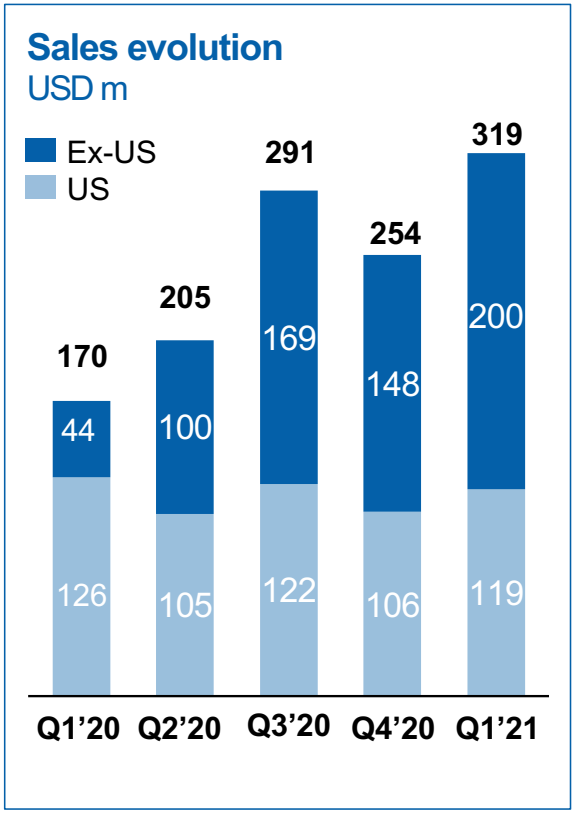
- US: strong demand; NBRx >6.2k drivers include expanded label, guideline updates[1]
- China: sales more than doubled (vs. Q1 2020), now second largest market
- EU: sales +22% (vs. Q1 2020)

Confident in future growth

- ~15% of eligible US CHF patients currently treated[2]
- Expanded US label strengthens essential role of Entresto® across HF continuum
- Hypertension indication (Asia)

NBRx – New-to-brand Prescriptions PCP – Primary Care Physician HF – Heart Failure 1. IQVIA National Prescription Audit 2. Only ~30% of eligible rEF patients currently treated in G7. Eligible patients defined as prevalent HFrEF patients within each market's label. G7 = US, CA, JP, DE, FR, IT, UK. 2. IMS National Prescription Audit

Zolgensma® strong quarter (USD 319m) driven by ongoing geographic expansion



Geographic expansion

- Strong growth ex-US from expanding access in Europe
- Stable US business driven by incident patients
- 1.2k patients have been treated with Zolgensma® worldwide[1]
- Improving newborn screening: target >80% in US, 20% in EU by end 2021[2]

New data[3] reinforce Zolgensma® as unique one-time therapy for SMA

- Age-appropriate development when used pre-symptomatically[3]
- Durability now 5+ years post-treatment[3]
- Ph3 SMART trial to strengthen confidence in children up to 21kg in EU

Continued commitment in gene therapy

- IT preclinical studies on track; pivotal study to be initiated after hold is lifted
- 10+ early-stage programs with two INDs planned in 2021

1. Commercially, via managed access programs and in clinical trials 2. Implementation may be impacted by COVID-related delays 3. MDA and AAN 2021

Kesimpta® uniquely positioned to become a first-choice treatment. Launch on track

High efficacy

Kappos L, et al
AAN 2021

ASCLEPIOS

Disability progression independent of relapse activity (PIRA) is common and often overlooked

Kesimpta® reduced PIRA vs. teriflunomide by:

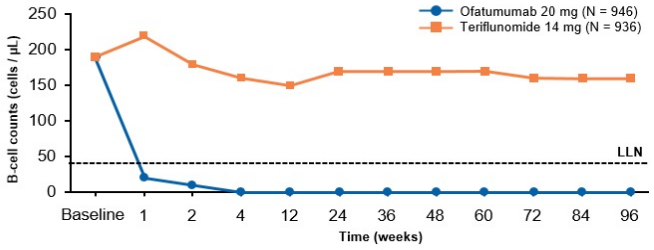
- 44-47% across broad RMS population
- **45-59%** in **newly diagnosed and treatment naive**[1]

Sustained B-cell depletion

without re-bound due to monthly dosing

ASCLEPIOS

Median B-cell counts over 96 weeks[2]



IgG levels preserved over 3 years[3]

ALITHIOS

Needed for immune defense against infections including COVID-19

Launch on track in US

- **USD 50m** sales includes USD 9m revenue adjustment relating to Q4 2020
- **~2.4k** patients treated, 51% naive or first switch[4]
- **>158m (~75%)** preferred commercial access[5]
- **Leading share of attention** with in-person / virtual meetings[6]
- Continuing market disruption by COVID-19 with **patient flow** reduced by 15%[7]

Approved in EU / JP

IgG - Immunglobulin G 1. Based on post-hoc data from the ASCLEPIOS trials. Kappos L, Montalban X, Coyle P, et al. Ofatumumab reduces disability progression independent of relapse activity in patients with relapsing multiple sclerosis. ePoster presentation at Virtual AAN Meeting; April 2021 2. Hauser et al., AAN 2020, B-cell Depletion and Efficacy Outcomes with Ofatumumab: Subgroup Analysis from the Pooled Phase 3 ASCLEPIOS I and II Trials; P7.1-013 For other references, please see slides 48-49

Leqvio®: response to CRL on track to submit Q2-Q3, expecting gradual launch ramp in Europe

Significant unmet need

18 m	Lives lost globally annually due to CVD – more than all cancers combined[1]
⇧	After decades of decline, no. of lives lost is rising again[2]
~60 m	Patients with ASCVD in US and EU[3]
~15%	Patients with LDL-C below 70 mg/dl, in the US and EU[4,5,6]
90%	On medium to high dose statin +/- ezetimibe in the US and EU[7,8]
<1%	Penetration of non-statin lipid lowering therapies available, in US, EU[7,8]

Shortcomings of available therapies

Clinical and non-clinical

Efficacy	High dose statin +/- ezetimibe not enough for patients with high LDL-C[4,5,6]
Adherence	365 pills / year with statins and ezetimibe, up to 26 injections / year with PCSK9i. Lack of adherence drives CV deaths and costs[9]
Safety	≥7% treated patients are intolerant to statins[10,11,12]
Access	Barriers to access for PCSK9i mAb limiting their patient uptake[13]
Affordability	Leading to >40% of patients discontinuing treatment with PCSK9i mAb after 6 months[9,13]

Updates

US

- Response to CRL to be submitted Q2-Q3 2021
- Pre-launch focus on health systems readiness

Europe

- **UK** on track for Q3 launch with NHS
- **ORION-4** readout expected 2026 due to COVID-19

For references, please see slides 48-49

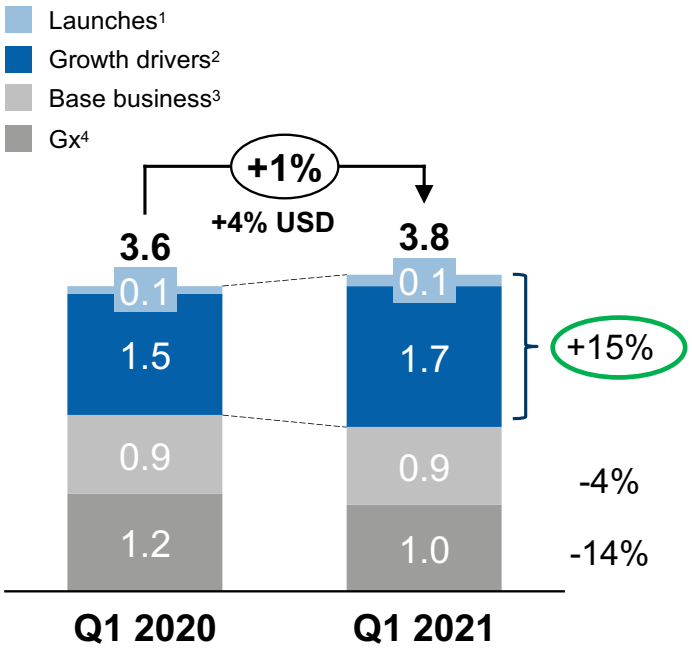
Susanne Schaffert

President, Novartis Oncology

Oncology net sales in Q1 broadly in line with PY, as strong growth portfolio offset Gx impact

Oncology net sales

USD bn, % cc



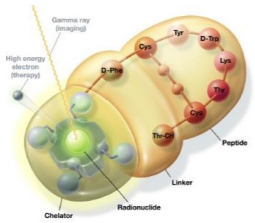
Solid growth, adjusting for PY forward purchasing

- Growth drivers and launches 48% of sales (up from 42% Q1 2020)
- Key drivers:
  - Kymriah® (USD 151m, +55%)
  - Promacta®/Revolade® (USD 463m, +13%)
  - Kisqali® (USD 195m, +19%)
  - Jakavi® (USD 363m, +8%)
- Diagnosis and treatment rates remain below pre-pandemic levels in key segments (e.g. breast cancer)
- Ongoing Gx impact

All growth % in cc 1. Launches include Piqray®, Adakveo® and Tabrecta® 2.Growth drivers include Promacta®/Revolade®, Tafinlar®+ Mekinist®, Kisqali®, Lutathera®, Kymriah® and Jakavi® (marketed by Novartis ex-US) 3. Base business – other brands 4. Gx include Afinitor®, Exjade®/Jadenu® , Glivec® and Sandostatin®

Strong foundation for our radioligand therapy platform

Scientific promise of RLT

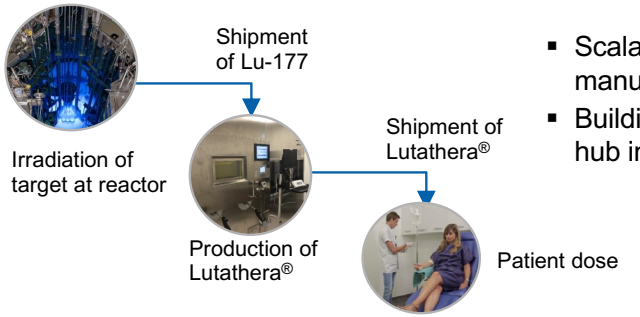


- Ability to target cytotoxic radiation directly to a tumor, limiting damage to surrounding healthy tissue
- Can use the same targeting compound labeled with either an imaging or therapeutic radionuclide
- Potential to innovate on the targeting compound, the radioisotope, and combinations, to address a wide range of cancers

Building platform and pipeline

2018	AAA	Acquisition, introduced new platform, brought in Lutathera®
	ENDOCYTE	Acquisition, expanded platform, added ^{177}Lu-PSMA-617
2021	itheranostics	Acquisition of FAP-targeting assets, to further deepen pipeline
	artios	Collaboration to explore novel DDR / RLT combinations
	AKTIS ONCOLOGY	Investment to develop alpha-based RLTs

Manufacturing capability developed



- Scalable and responsive manufacturing network
- Building major manufacturing hub in Indianapolis

Strong global commercial experience

- Lutathera® Q1 sales of USD 122m, +6% cc vs PY
- **> 9,000** patients treated since US/EU launch
- **> 400** centers actively treating patients globally
- Early rapid uptake in NET centers of excellence
- Current focus on increasing access at the community level, particularly in the US

^{177}Lu-PSMA-617 met both primary endpoints in Ph3 VISION study, improving OS and rPFS for advanced prostate cancer patients

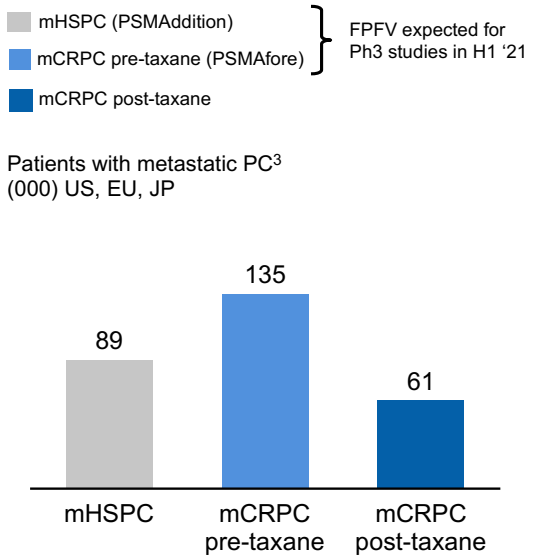
Significant unmet need in prostate cancer

2nd	Most diagnosed cancer in men
>80%	Patients metastatic at the time of CRPC diagnosis
<15%	5-year survival prognosis for mCRPC patients
~10	Months median overall survival[1]

VISION study positive for 3/4L mCRPC; submission expected H2 2021

- **^{177}Lu-PSMA-617 met both primary endpoints** of OS and rPFS vs. best standard of care
- **Patient population:** PSMA+ mCRPC patients, who have had previous taxane therapy (1-2 regimens) and ARDTs (≥1 regimen); >80% of prostate cancer patients express PSMA
- **Data to be presented** at an upcoming medical congress
- **Submission on track** for H2 2021
- **Pre-launch activities on track**, focus on: community centers, PSMA awareness, site capacity expansion

Moving ^{177}Lu-PSMA-617 into earlier lines of therapy

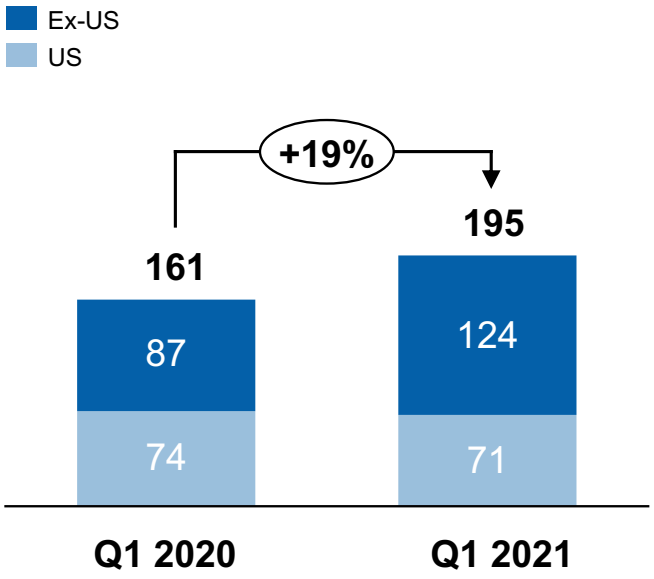


mCRPC – Metastatic castration resistant prostate cancer PSMA – Prostate specific membrane antigen mHSPC – Metastatic hormonal sensitive prostate cancer For references, please see slides 48-49

Kisqali® grew 19% in Q1, with solid performance and share gains ex-US

Sales evolution

USD m, % cc



Kisqali® provides clear differentiation in CDK4/6 class

- Longest reported median OS among all Ph3 trials in aBC, reaching ~5 years in pre-menopausal patients
- Unique profile increasingly recognized by payers: Kisqali® only CDK4/6 routinely reimbursed by UK NHS in 2L aBC

Solid growth despite COVID-19 impact on CDK4/6 market

- Ex-US: Strong double-digit growth, driven by continued patient share uptake in EU4, UK and further geographic expansion
- US: Maintained NBRx/TRx share in a declining market[1]

Confident in future growth

- Expect growth to accelerate as pandemic eases, particularly in US
- NATALEE adjuvant study completed enrollment; readout expected 2022

aBC – Advanced breast cancer 1. IQVIA: Total CDK4/6 market NBRx -16% in Feb vs PY, TRx – 4% in Feb vs PY

Harry Kirsch
Chief Financial Officer

Financial review and 2021 guidance

Q1 operational performance impacted by PY forward purchasing and continuation of COVID-19

Group USD million	Q1 2021	Change vs. PY % USD	Change vs. PY % cc[1]
Net Sales	12,411	1	-2
Core Operating Income[1]	3,957	-5	-8
Operating Income	2,415	-12	-14
Net Income	2,059	-5	-7
Core EPS (USD)[1]	1.52	-3	-5
EPS (USD)	0.91	-5	-6
Free Cash Flow[1]	1,597	-21	

Impact from PY forward purchasing[2]

Sales -3% pts

Core OpInc -7% pts

Free cash flow

Including USD 650m upfront payment for tislelizumab in-licensing

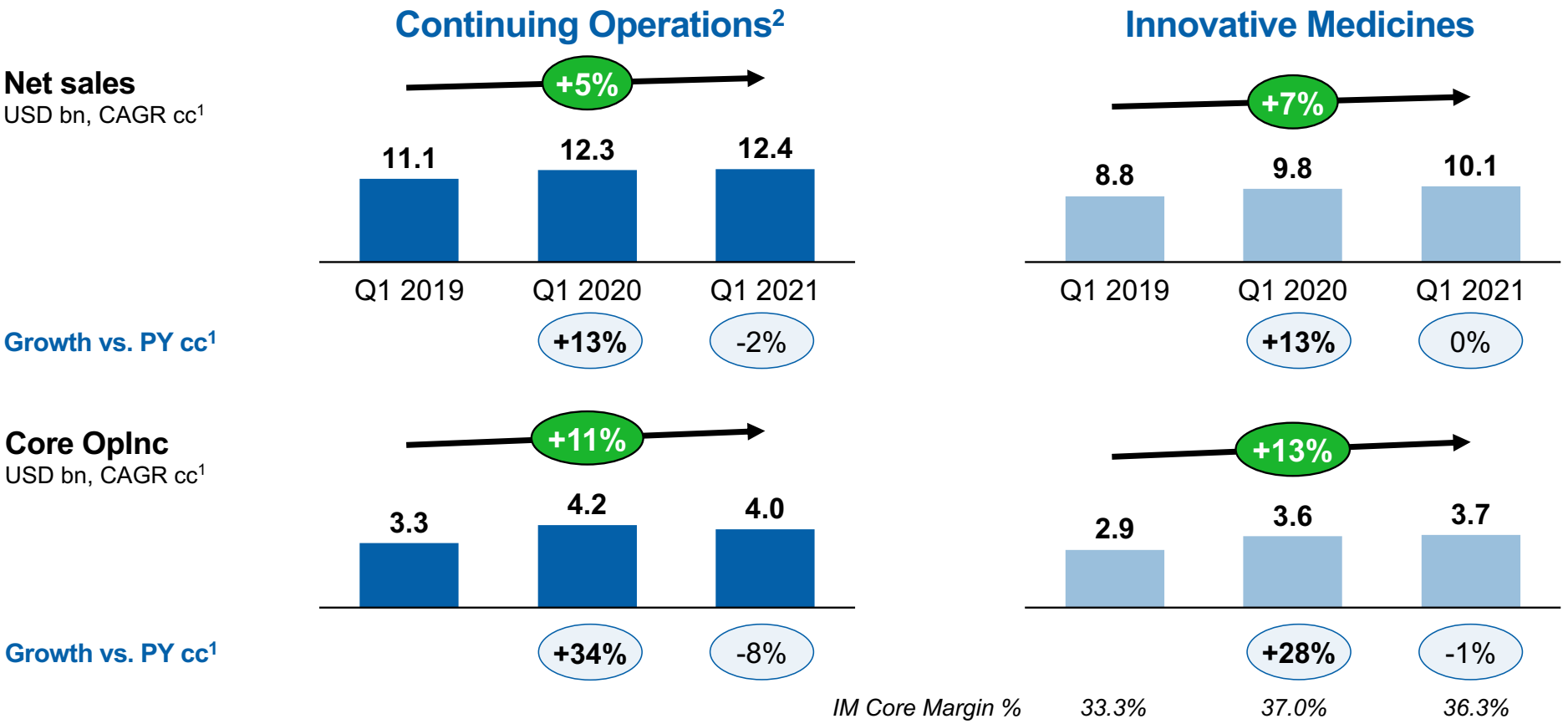
1. Constant currencies (cc), core results, free cash flow are non-IFRS measures. An explanation of non-IFRS measures can be found on page 36 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates in this Release refer to same period in prior year 2. Growth excluding prior year COVID-19 related forward purchasing is a non-IFRS measure, an explanation for this measure can be found on page 44 of the Condensed Interim Financial Report

Excluding PY forward purchasing, solid Innovative Medicines division performance

	Q1 2021				Q1 2021 ex. PY forward purchasing		
	Net sales change vs. PY (in % cc)[1]	Core operating income change vs. PY (in % cc)[1]	Core margin (%)[1]	Core margin change vs. PY (%pts cc)[1]	Net sales change vs. PY (in % cc)[1,2]	Core operating income change vs. PY (in % cc)[1,2]	Core margin change vs. PY (%pts cc)[1,2]
Innovative Medicines	0	-1	36.3	-0.5	3	6	1.0
Sandoz	-13	-35	19.3	-6.8	-9	-29	-5.7
Group	-2	-8	31.9	-1.8	1	-1	-0.4

1. Constant currencies (cc), core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 36 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates in this Release refer to same period in prior year 2. Growth excluding prior year COVID-19 related forward purchasing is a non-IFRS measure, an explanation for this measure can be found on page 44 of the Condensed Interim Financial Report

Strong operational performance over 2 years

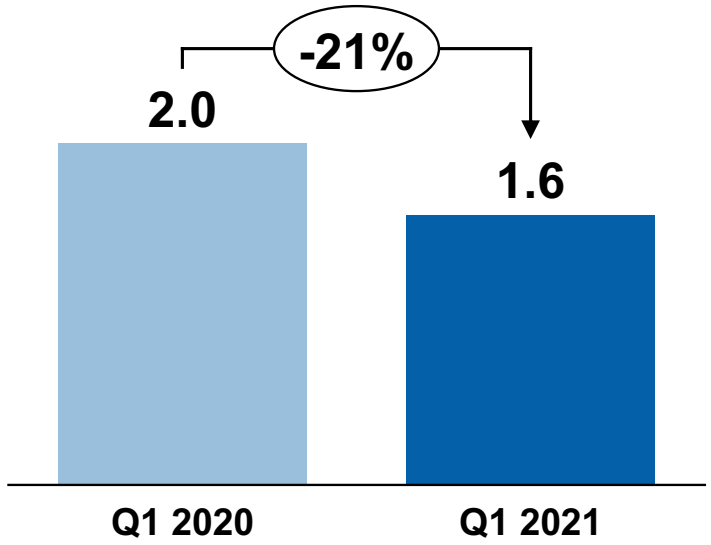


1. Constant currencies (cc), core results are non-IFRS measures. An explanation of non-IFRS measures can be found on page 36 of the Condensed Interim Financial Report. Unless otherwise noted, all growth rates in this Release refer to same period in prior year 2. Continuing operation include the business of Innovative Medicines, Sandoz and continuing corporate functions

Q1 2021 free cash flow decreased to USD 1.6bn

Group free cash flow[1]

USD bn



Key drivers vs. PY:

– Tislelizumab in-licensing (upfront payment USD 650m)

– Lower operating income (adjusted for non-cash items)

\+ Favorable changes in working capital

1. Constant currencies (cc), core results and free cash flow are non-IFRS measures. An explanation of non-IFRS measures can be found on page 36 of the Condensed Interim Financial Report.

2021 Novartis full year guidance

Barring unforeseen events; growth vs. PY in cc

Group | full year guidance[1]

vs. PY (cc)

Group Sales expected to grow **low to mid single digit**

- **IM Division** expected to **grow mid single digit**
- **Sandoz** expected to **decline low to mid single digit** (revised from broadly in line)

Group Core operating income expected to grow **mid single digit, ahead of sales**

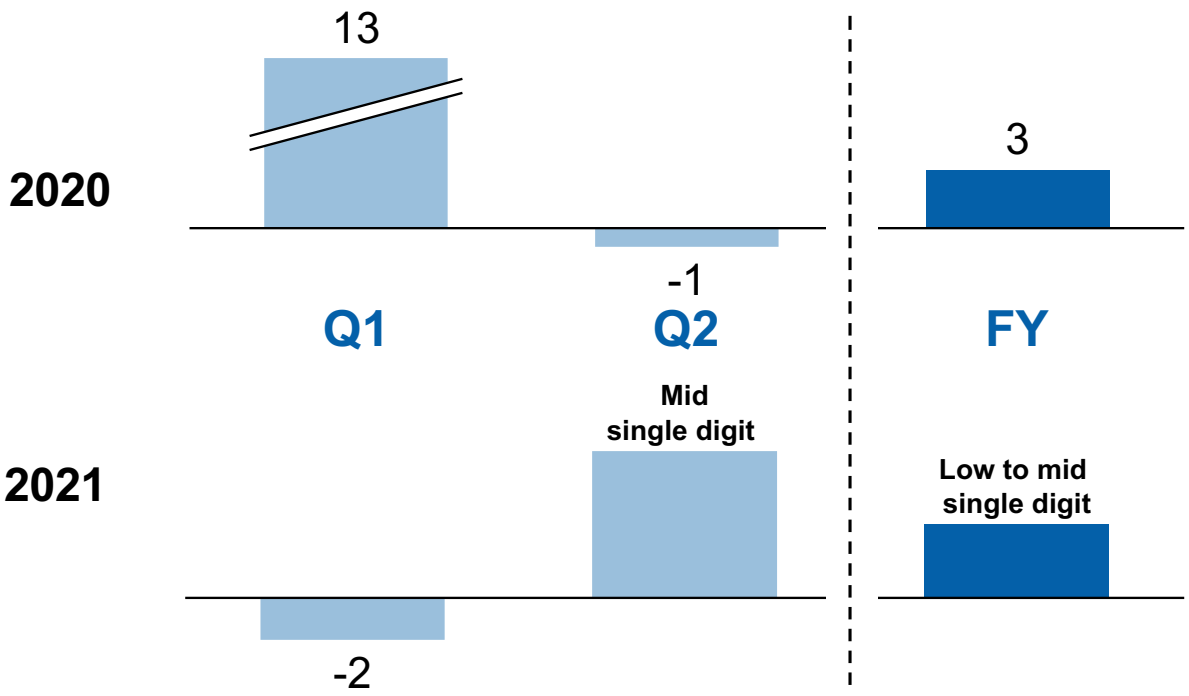
- **IM Division** expected to **grow mid to high single digit, ahead of sales**
- **Sandoz** expected to **decline low to mid teens**

1. Key assumptions:
   - Our guidance assumes that we see a return to normal global healthcare systems including prescription dynamics by mid 2021
   - In addition, we assume that no Gilenya® and no Sandostatin® LAR generics enter in 2021 in the US

Q2 2021 sales expected to grow mid single digit benefiting from PY COVID-19 forward purchasing reversal

Group sales growth vs. PY

%pts, cc



Sales

Q1 2021: broadly in line vs. PY ex. forward purchasing ✓

Q2 2021: growth benefiting from approximately 3%pts of PY forward purchasing reversal

Core operating income

H1 2021: expected to decline low single digit due to PY low cost base and investments to support H2 growth

Key assumption:
Return to normal global healthcare systems including prescription dynamics by mid 2021

Vas Narasimhan

Chief Executive Officer

2021 catalysts maintaining long-term momentum

Potential catalysts	Selected examples			
Major approvals	**Kesimpta® (EU/JP)** ✓ RMS	**Entresto® (US)** ✓ HFpEF	**Cosentyx® (US/JP/CN)** Pediatric psoriasis	
Major submissions[1]	**Alpelisib (BYL719)** PROS	**Asciminib (ABL001)** CML	**Jakavi®** ✓ Acute and chronic GvHD	**Beovu®** DME
	^{177}Lu-PSMA-617 mCRPC	**Kymriah®** FL	**Leqvio® (US)[2]** Hyperlipidemia	**Tislelizumab (VDT482)** Esophageal cancer, NSCLC
Major readouts Enabling submission 2021	**Kymriah®** r/r DLBCL 1st relapse	**Sabatolimab (MBG453)** MDS	**Canakinumab (ACZ885)[3]** NSCLC 1L	**Entresto®[4]** ✓ Post-AMI
Enabling submission 2022	**Ligelizumab (QGE031)[5]**CSU	**Cosentyx®** HS		
Others	**Iptacopan (LNP023)** ✓ Ph2 IgAN[6]	**Iptacopan (LNP023)** ✓ Ph2 PNH[7]	**Iptacopan (LNP023)** Ph2 C3G	**Kisqali®** Breast cancer (MONALEESA)
Pivotal study starts	**Iptacopan (LNP023)** ✓ Ph3 IgAN	**Iptacopan (LNP023)** ✓ Ph3 C3G	**Iptacopan (LNP023)** Ph3 aHUS	**Ligelizumab (QGE031)** Food allergy
	Ligelizumab (QGE031) CINDU	**177Lu-PSMA-617** pre-taxane	**177Lu-PSMA-617** mHSPC	

1. First submission in any market. 2. Novartis received a CRL from the FDA due to unresolved facility inspection-related conditions at a third-party manufacturing facility in Europe. FDA has not raised any concerns related to the efficacy or safety of inclisiran. Response to CRL planned to be submitted Q2 - Q3 2021. 3. Depending on timing of final read-out submission may move to early 2022 4. Numerical trends consistently favored Entresto® vs. active comparator but did not meet primary composite endpoint. The safety profile of Entresto® was confirmed 5. Q4/2021-Q1/2022 potential COVID impact 6. IgAN Ph2 data to be published at upcoming medical meeting 7. Ph2 in PNH anti-C5 treatment naive patients

Conclusion Q1

Growth drivers and launches continued their strong momentum

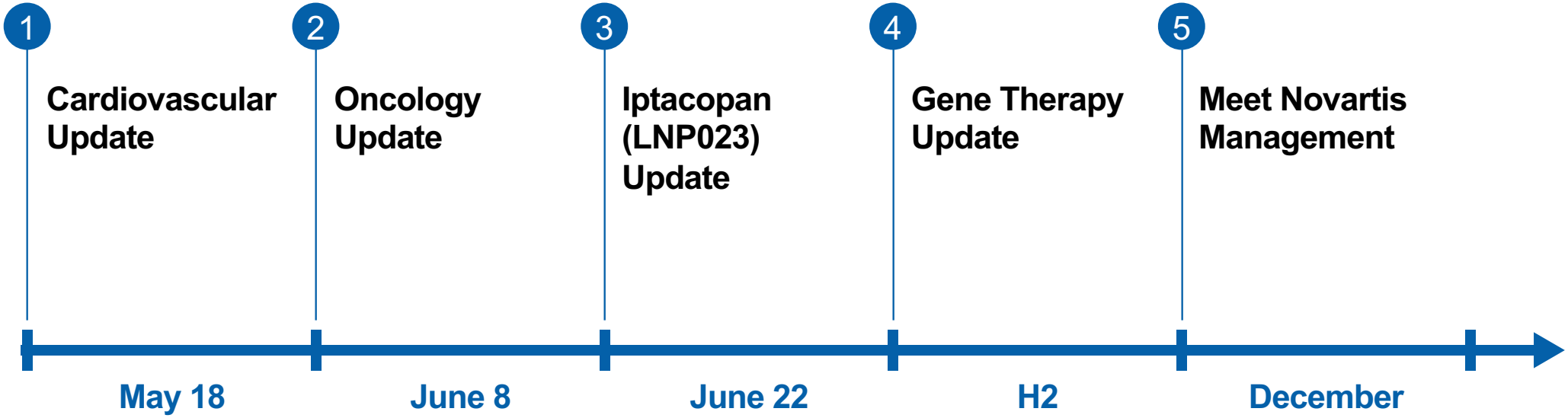
Solid IM top and bottom line performance, PY forward purchasing making tough comps

Progressing our broad pipeline of novel medicines

Confident in delivering our 2021 and longer term growth outlook

Novartis planned data-related events in 2021

Events

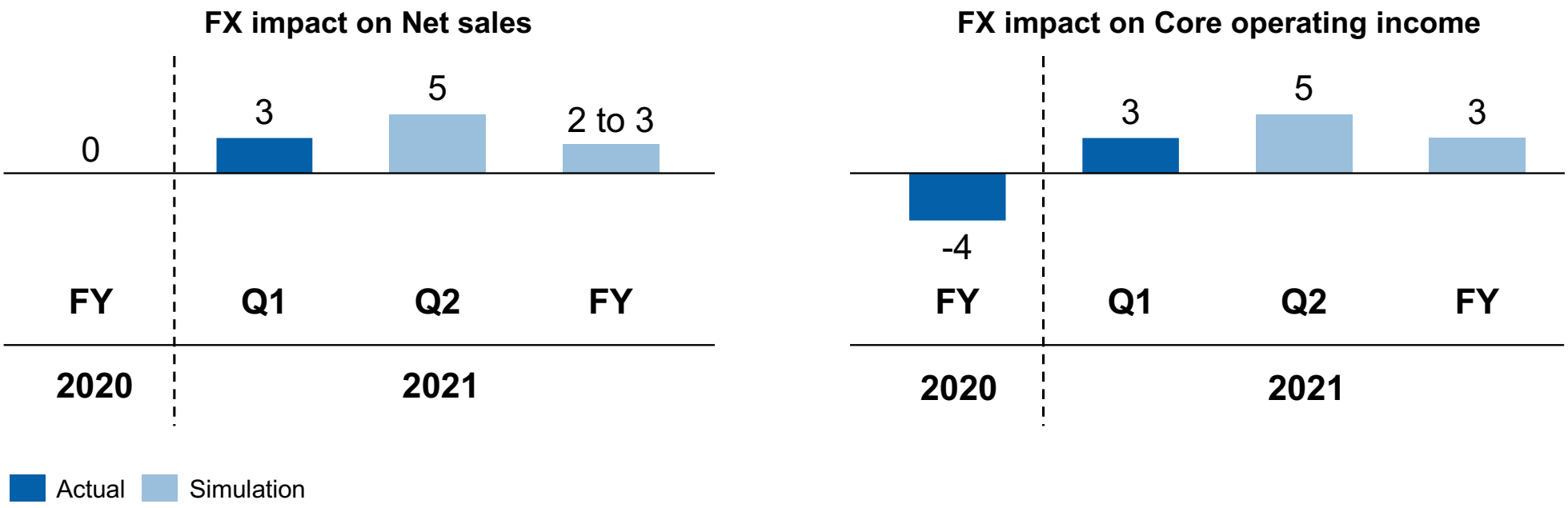


Appendix

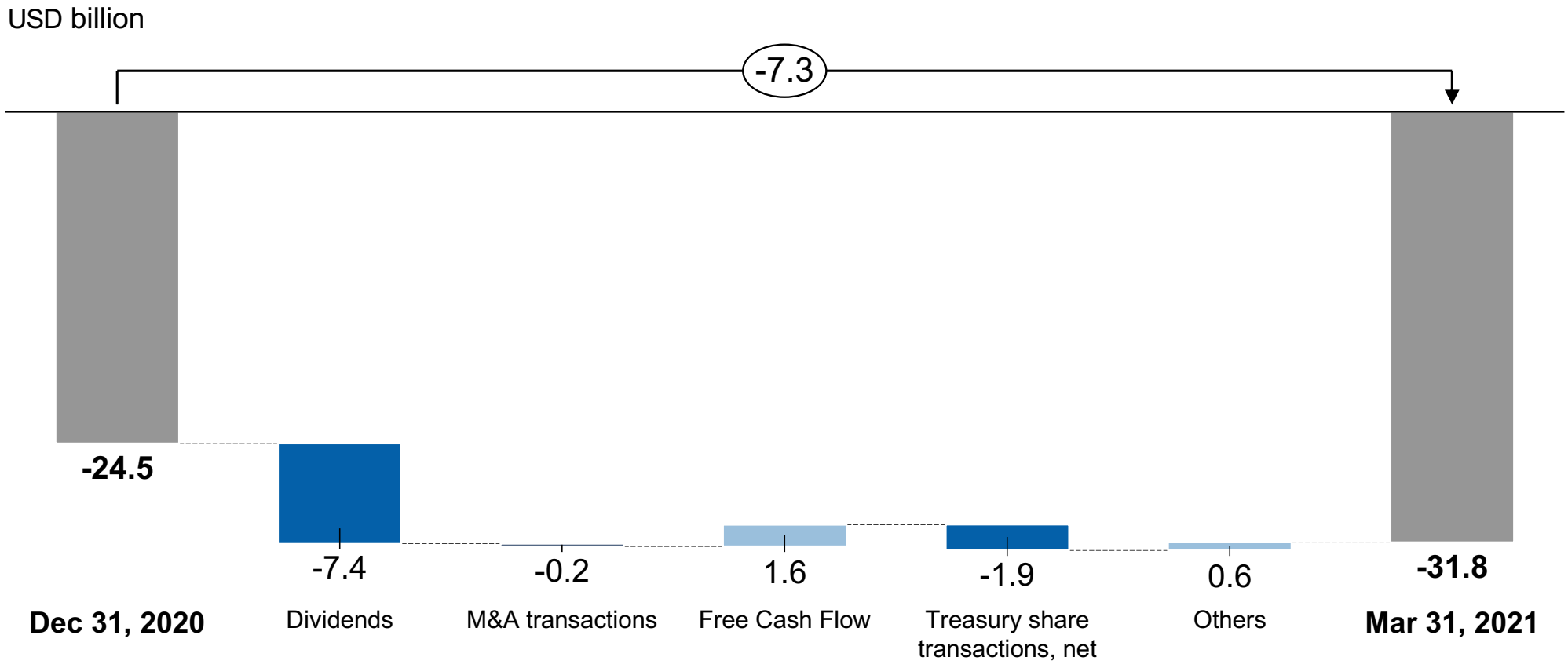
Expected currency impact for Q2 and full year 2021

Currency impact vs. PY

%pts, assuming late-April exchange rates prevail in 2021



Net debt increased by USD 7.3bn mainly due to the annual dividend payment and share buybacks



2021 key pipeline milestones[1]

✓ Achieved ✗ Missed

	H1 2021			H2 2021	
Regulatory decisions and opinions	Entresto®	HFpEF (US)	✓	Cosentyx®	Pediatric psoriasis (US / CN / JP)
	Kesimpta®	Relapsing MS (EU / JP)	✓		
Major expected submissions	Leqvio®	Hyperlipidemia (US)[2]		Asciminib (ABL001)	CML 3L (JP)
	Jakavi®	Acute and chronic GvHD (EU / JP)	✓	Beovu®	DME (JP)
	Tabrecta®	NSCLC (EU)	✓	Alpelisib (BYL719)	PROS (US)
	Beovu®	DME (US / EU)		Kymriah®	r/r Follicular lymphoma (US/EU/JP)
	Asciminib (ABL001)	CML 3L (US /EU)		^{177}Lu-PSMA-617	Ph3 – mCRPC (US/EU)
				Tislelizumab (VDT482)	Esophageal cancer (US)
				Tislelizumab (VDT482)	NSCLC (US)
Major expected trial readouts*	Iptacopan (LNP023)	Ph2 - IgAN	✓ [3]	Canakinumab (ACZ885)	Ph3 - NSCLC 1L
	Iptacopan (LNP023)	Ph2 - C3G		ECF843	Ph2 - Dry eye
	Entresto®	Ph3 - Post-AMI	✓ [5]	Ligelizumab (QGE031)	Ph3 – CSU[4]
	Canakinumab (ACZ885)	Ph3 - NSCLC 2L	✓ [6]	Kisqali®	aBC (MONALEESA-2 OS)
	^{177}Lu-PSMA-617	Ph3 - mCRPC	✓	Remibrutinib (LOU064)	Ph2 - CSU
	Cosentyx®	Ph3 - JIA	✓	Cosentyx®	Ph3 - HS
				Sabatolimab (MBG453)	Ph2, MDS
				Kymriah®	Ph3, r/r DLBCL 1st relapse

*Achieved = on-time readout of data, irrespective of trial outcome. 1. 2021 Key milestone table may evolve based on read-out outcomes as well as BD&L activities 2. Novartis received a CRL from the FDA due to unresolved facility inspection-related conditions at a third-party manufacturing facility in Europe. FDA has not raised any concerns related to the efficacy or safety of inclisiran. Response to CRL planned to be submitted Q2 - Q3 2021 3. IgAN Ph2 data to be published at upcoming medical meeting 4. Q4/2021-Q1/2022 potential COVID impact 5. Numerical trends consistently favored Entresto® vs. active comparator but did not meet primary composite endpoint. The safety profile of Entresto® was confirmed 6. Negative readout

Our pipeline projects at a glance

	Phase 1/2	Phase 3	Registration	Total
ONCOLOGY	50	21	3	74
PHARMACEUTICALS	61	24	2	87
Cardiovascular, Renal, Metabolism	8	7	1	16
Immunology, Hepatology, Dermatology	26	9	1	36
Neuroscience	7	2	0	9
Ophthalmology	6	3	0	9
Respiratory	8	2	0	10
Global Health	6	1	0	7
BIOSIMILARS	0	1	0	1
Total	**111**	**46**	**5**	**162**

Novartis pipeline in Phase 1 (1 of 2)

38 lead indications

Lead indication

Oncology						
Code	**Name**	**Mechanism**	**Indication(s)**			
AAA603	^{177}Lu-NeoB	Radioligand therapy target GRPR	Multiple solid tumors			
AAA602	^{177}Lu-PSMA-R2	Radioligand therapy target PSMA	Prostate cancer			
ADPT01	ADPT01	-	TNBC (combos)	Colorectal cancer (combos)		
ADPT03	ADPT03	BCL11A	Sickle cell anemia			
CSJ137	CSJ137	Growth factor inhibitor	Anaemia			
CTL019	Kymriah®	CD19 CART	Lymphoma			
DKY709	DKY709 + spartalizumab	Novel immunomodulatory agent	Cancers			
EGF816	nazartinib + LXH254, ribociclib, capmatinib, opdivo, mekinist	EGFR inhibitor	NSCLC (combo)			
HDM201	HDM201 + MBG453, venetoclax	MDM2 inhibitor	Haematological malignancy			
JBH492	JBH492	-	Haematological malignancy			
JDQ443	JDQ443	KRAS Inhibitor	Solid tumors			
JEZ567	JEZ567	CD123 CART	AML			
KAZ954	KAZ954	-	Solid tumors			
LHC165	LHC165 + spartalizumab	TLR7 agonist	Solid tumors			
LXF821	LXF821	EGFR CART	Glioblastoma multiforme			
LXH254	LXH254 (combos)	cRAF inhibitor	Solid tumors			
MAK683	MAK683	EED inhibitor	Cancers			
MCM998	MCM998, LXG250	BCMA CART, CD19 CART	Multiple myeloma			
MIK665	MIK665	MCL1 inhibitor	AML (combo)			
NIS793	NIS793, spartalizumab	TGFB1 inhibitor	Solid tumors			
NIZ985	NIZ985, spartalizumab	IL-15 agonist	Solid tumors			
NZV930	NZV930, spartalizumab, NIR178	CD73 antagonist	Solid tumors			
PDR001	spartalizumab (combos)	PD1 inhibitor	AML	Solid tumors (combo)		
PHE885	PHE885	BCMA cell therapy	Haematological malignancy			
SQZ622	SQZ622	CD123xCD3 modulator	AML			
TNO155	TNO155	SHP2 inhibitor	Solid tumors (single agent)	Solid tumors (combo)	Solid tumors (combo)	
VAY736	ianalumab + ibrutinib	BAFF-R inhibitor	Haematological malignancy			
VOB560	VOB560	-	Cancers			
VPM087	gevokizumab	IL1B Antagonist	CRC 1st line			
WNT974	WNT974 + spartalizumab	Porcupine Inhibitor	Solid tumors			
WVT078	WVT078	-	Multiple myeloma			
YTB323	YTB323 ± ibrutinib	CD19 CART	Haematological malignancy			

Novartis pipeline in Phase 1 (2 of 2)

38 lead indications

Lead indication

Immunology, Hepatology, Dermatology

Code	Name	Mechanism	Indication(s)
CEE321	CEE321	Pan JAK Inhibitor	AD
DFV890	DFV890	-	Anti-inflammatory therapy
FIA586	FIA586	-	NASH
MHS552	MHS552	-	Autoimmune indications
MHV370	MHV370	-	Sjögren's, SLE
NGI226	NGI226	-	Tendinopathy

Neuroscience

Code	Name	Mechanism	Indication(s)
OAV201	OAV201 (AVXS-201)	MECP2 gene therapy	Rett syndrome
NIO752	NIO752	Tau antagonist	Neurodegenerative diseases
LMI070	branaplam	mRNA splicing modulator	Huntington

Ophthalmology

Code	Name	Mechanism	Indication(s)
MHU650	MHU650	-	Diabetic eye diseases

Respiratory Disease

Code	Name	Mechanism	Indication(s)
LTP001	LTP001	-	Respiratory diseases
NCJ424	NCJ424	-	Respiratory diseases

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)
MBL949	MBL949	-	Obesity related diseases

Global Health

Code	Name	Mechanism	Indication(s)
KAF156	ganaplacide	-	Malaria prophylaxis

Novartis pipeline in Phase 2

30 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)			
BYL719	alpelisib	PI3Kα inhibitor	PROS			
BLZ945	BLZ945	CSF-1R Inhibitor	Solid tumors			
DRB436	Tafinlar® + Mekinist®	BRAF inhibitor + MEK inhibitor	HGG/LGG - Pediatrics			
INC280	capmatinib	Met inhibitor	Solid tumors	NSCLC (Combo)		
INC424	Jakavi®	JAK1/2 inhibitor	Myelofibrosis (combination)		Pediatrics acute GVHD	Pediatrics chronic GVHD
LXH254	LXH254	cRAF inhibitor	Melanoma (combo)			
MBG453	sabatolimab	TIM3 antagonist	Unfit AML			
NIR178	NIR178, spartalizumab	Ad2AR inhibitor, PD1 inhibitor	Cancers			
NIS793	NIS793	TGFB1 inhibitor	Pancreatic cancer			
PDR001	spartalizumab	PD1 inhibitor	Metastatic melanoma (combo)			
SEG101	crizanlizumab	P-selectin Inhibitor	Ped sickle cell anaemia with crisis			

Immunology, Hepatology, Dermatology

Code	Name	Mechanism	Indication(s)			
ADPT02	ADPT02	-	NASH (Combos)			
AIN457	Cosentyx®	IL17A inhibitor	GCA	Lichen planus		
CFZ533	iscalimab	CD40 inhibitor	Renal Tx	Sjögren's	HS	Liver Tx
LJN452	tropifexor + licogliflozin	FXR agonist	NASH (combos)			
LNA043	LNA043	ANGPTL3 agonist	Osteoarthritis			
LOU064	remibrutinib	BTK inhibitor	CSU	Sjögren's		
LRX712	LRX712	-	Osteoarthritis			
LYS006	LYS006	Anti-inflammatory	Acne	Colitis ulcerative	HS	
MAS825	MAS825	-	NLRC4-GOF indications			
VAY736	ianalumab	BAFF-R inhibitor	Sjögrens	AIH	SLE	

Ophthalmology

Code	Name	Mechanism	Indication(s)			
CPK850	CPK850	RLBP1 AAV	RP			
ECF843	ECF843	rh-Lubricin	Dry eye			
LKA651	LKA651	EPO inhibitor	DME			
SAF312	SAF312	TRPV1 antagonist	COSP			
UNR844	UNR844	Disulfide bonds modulator	Presbyopia			

Neuroscience

Code	Name	Mechanism	Indication(s)			
BLZ945	BLZ945	CSF-1R Inhibitor	ALS			
LMI070	branaplam	mRNA splicing modulator	SMA			
MIJ821	MIJ821	NR2B Inhibitor	Depression			
OAV101	AVXS-101	Survival motor neuron (SMN) gene therapy	SMA IT[1)]			

Respiratory Disease

Code	Name	Mechanism	Indication(s)			
CMK389	CMK389	IL-18 inhibitor	Pulmonary sarcoidosis			
CSJ117	CSJ117	TSLP inhibitor	Asthma			
DFV890	DFV890	-	COVID-19 related pneumonia			
MAS825	MAS825	-	COVID-19 related pneumonia			
QBW251	icenticaftor	CFTR potentiator	COPD	Bronchiectasis		

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)			
CFZ533	iscalimab	CD40 inhibitor	Lupus nephritis	T1DM		
HSY244	HSY244	-	Atrial fibrillation			
LMB763	nidufexor	FXR agonist	Diabetic nephropathy			
LNP023	iptacopan	CFB inhibitor	C3G	iMN	aHUS	

Global Health

Code	Name	Mechanism	Indication(s)			
AFQ056	AFQ056	mGluR5 Antagonist	Cocaine use disorder			
KAE609	cipargamin	PfATP4 inhibitor	Malaria severe	Malaria uncomplicated		
KAF156	ganaplacide	-	Malaria uncomplicated			
LXE408	LXE408	Protozoan inhibitor	Visceral leishmaniasis			

1. Preclinical studies to address partial clinical hold are on track

Novartis pipeline in Phase 3

7 lead indications

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)			
AAA617	^{177}Lu-PSMA-617	Targeted radioligand therapy	mCRPC	mCRPC pre-taxane		mHSPC
AAA601[1]	Lutathera®	Targeted radioligand therapy	GEP-NET 1L G3			
ABL001	asciminib	BCR-ABL inhibitor	CML 3L			
ACZ885	canakinumab	IL-1b inhibitor	NSCLC 1L	Adjuvant NSCLC		
BYL719	Piqray®	PI3Kα inhibitor	HER2+ adv BC	TNBC	HNSCC 2/3L	Ovarian cancer
CTL019	Kymriah®	CD19 CART	r/r Follicular lymphoma	1L high risk ALL, pediatrics and young adults	r/r DLBCL 1st relapse	
DRB436	Tafinlar® + Mekinist®	BRAF inhibitor + MEK inhibitor	Thyroid cancer			
ETB115	Promacta®	Thrombopoietin receptor (TPO-R) agonist	Radiation sickness syndrome		Food effect free formulation	
LEE011	Kisqali®	CDK4/6 Inhibitor	HR+/HER2- BC (adj)			
MBG453	Sabatolimab	TIM3 antagonist	HR-MDS			
VDT482	tislelizumab	PD1 Inhibitor	Esophageal cancer	NSCLC	*Multiple indications*	

Immunology, Hepatology, Dermatology

Code	Name	Mechanism	Indication(s)		
AIN457	Cosentyx®	IL17A Inhibitor	Lupus Nephritis	Juvenile idiopathic arthritis	AS H2H
			IV regimen in PsA	IV regimen in Axial SpA	HS
QGE031	ligelizumab	IgE Inhibitor	CSU	CINDU	Food allergy

Ophthalmology

Code	Name	Mechanism	Indication(s)		
RTH258	Beovu®	VEGF Inhibitor	Diabetic retinopathy	RVO	DME

Neuroscience

Code	Name	Mechanism	Indication(s)			
AMG334	Aimovig®	CGRPR antagonist	Ped Migraine			
BAF312	Mayzent®	S1P1,5 receptor modulator	Ped MS			

Respiratory Disease

Code	Name	Mechanism	Indication(s)			
IGE025	Xolair®	IgE inhibitor	Food allergy	Auto-injector		

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)			
KJX839	Leqvio®	siRNA (regulation of LDL-C)	CVRR-LDLC	Ped Hyperlipidemia		
LCZ696	Entresto®	Angiotensin receptor/neprilysin inhibitor	Post-AMI	Pediatric CHF[3]		
LNP023	Iptacopan	CFB inhibitor	PNH	IgAN		
TQJ230	Pelacarsen	ASO targeting Lp(a)	CVRR-Lp(a)			

Global Health

Code	Name	Mechanism	Indication(s)
COA566	Coartem®	-	Malaria uncomplicated, new formulation <5kg patients

Biosimilars

Code	Name	Mechanism	Indication(s)		
GP2411	denosumab	anti RANKL mAb	Denosumab BioS		

1. ^{177}Lu-dotatate in US 2. Approved in US & JP 3. Approved in US

Novartis pipeline in registration

1 lead indication

Lead indication

Oncology

Code	Name	Mechanism	Indication(s)	
INC424	Jakavi®	JAK1/2 inhibitor	Acute GVHD	Chronic GVHD
INC280	capmatinib	Met inhibitor	NSCLC EU[2)]	

Immunology, Hepatology, Dermatology

Code	Name	Mechanism	Indication(s)
AIN457	Cosentyx®	IL17A inhibitor	300 mg AI

Cardiovascular, Renal, Metabolism

Code	Name	Mechanism	Indication(s)			
KJX839	Leqvio®	siRNA (regulation of LDL-C)	Hyperlipidemia			

1. Approved in US as Kesimpta®

Novartis submission schedule

New Medical Entities: Lead and supplementary indications

LEAD INDICATIONS

2021	2022	2023	2024	≥2025		
177Lu-PSMA-617 Lead AAA617 mCRPC 3L	**ligelizumab** Lead QGE031 CSU	**ECF843** Lead Dry eye	**Icenticaftor** Lead QBW251 COPD	**^{177}Lu-NeoB** Lead AAA603 Multiple Solid Tumors	**iscalimab** Lead CFZ533 Renal Tx	**NIS793** Lead Solid tumors
asciminib Lead ABL001 CML 3L		**iptacopan** Lead LNP023 PNH	**SAF312** Lead COSP	**^{177}Lu-PSMA-R2** Lead AAA602 Prostate cancer	**ianalumab** Lead VAY736 Sjögren's syndrome	**OAV201** Lead AVXS-201 Rett syndrome
sabatolimab Lead MBG453 HR-MDS			**UNR844** Lead Presbyopia	**CEE321** Lead Atopic Dermatitis	**LMI070** Lead Huntington's disease	**pelacarsen** Lead TQJ230 CVRR-Lp(a)
tislelizumab Lead VDT842 Esophageal cancer				**cipargamin** Lead KAE609 Malaria severe	**LNA043** Lead Osteoarthritis	**remibrutinib** LOU064 CSU
				CPK850 Lead RP	**LXE408** Lead Visceral leishmaniasis	**spartalizumab** Lead PDR001 Malignant melanoma (combo)
				CSJ117 Lead Asthma	**LXH254** Lead Solid tumors	**TNO155** Lead Solid tumors
				ganaplacide Lead KAF156 Malaria uncomplicated	**mavoglurant** Lead AFQ056 Cocaine use disorder	**tropifexor&licogliflozin** NME LJN452 NASH (combos)
				gevokizumab Lead VPM087 1st line CRC / 1st line RCC	**MIJ821** Lead Depression	

NEW INDICATIONS

tislelizumab LCM VDT842 NSCLC		**177Lu-PSMA-617** LCM AAA617 Pre-taxane	**177Lu-PSMA-617** LCM AAA617 mHSPC	**cipargamin** LCM KAE609 Malaria uncomplicated	**iptacopan** LCM LNP023 aHUS	**LMI070** LCM SMA
		iptacopan LCM LNP023 C3G	**crizanlizumab** LCM SEG101 Sickle cell anaemia with crisis ped	**iscalimab** LCM CFZ533 Liver Tx	**ianalumab** LCM VAY736 AIH	**remibrutinib** LCM LOU064 Sjögren's syndrome
		iptacopan LCM LNP023 IgAN	**ligelizumab** LCM QGE031 CINDU	**iscalimab** LCM CFZ533 Sjögren's syndrome	**ligelizumab** LCM QGE031 Food allergy	
			sabatolimab LCM MBG453 Unfit AML	**iptacopan** LCM LNP023 iMN		

Novartis submission schedule

Supplementary indications for existing brands

2021[1)]	2022	2023	2024	≥2025		
alpelisib LCM BYL719 PROS	**Cosentyx** LCM secukinumab, AIN457 PsA IVIV	**canakinumab** LCM ACZ885 Adjuvant NSCLC	**Beovu** LCM brolucizumab, RTH258 RVO	**Aimovig** LCM erenumab, AMG334 Pediatric Migraine	**Leqvio** LCM KJX839 CVRR-LDLC	**Mayzent** LCM siponimod, BAF312 Pediatric MS
Beovu LCM brolucizumab, RTH258 DME	**Cosentyx** LCM secukinumab, AIN457 AS H2H	**Cosentyx** LCM secukinumab, AIN457 AS IVIV	**Coartem** LCM artemether + lumefantrine, CCA566 Malaria uncompl., formula for <5kg	**Beovu** LCM brolucizumab, RTH258 Diabetic retinopathy	**Jakavi** LCM ruxolitinib, INC424 Myelofibrosis (combination)	**Piqray** LCM alpelisib, BYL719 HNSCC 2/3L
canakinumab[1] LCM ACZ885 NSCLC 1L	**Cosentyx** LCM secukinumab, AIN457 Hidradenitis suppurativa	**Denosumab** BioS GP2411 anti RANKL mAb	**Cosentyx** LCM secukinumab, AIN457 GCA	**Cosentyx** LCM secukinumab, AIN457 Lichen Planus	**Jakavi** LCM ruxolitinib, INC424 Pediatrics Chronic GVHD	**Piqray** LCM alpelisib, BYL719 HER2+ adv BC
Cosentyx LCM secukinumab, AIN457 Juvenile idiopathic arthritis	**Entresto EU[3)]** LCM sacubitril/valsartan, LCZ696 Pediatric CHF	**Kisqali** LCM ribociclib, LEE011 HR+/HER2- BC (adj)	**Jakavi** LCM ruxolitinib, INC424 Pediatrics Acute GVHD	**Cosentyx** LCM secukinumab, AIN457 Lupus Nephritis	**Kymriah** LCM tisagenlecleucel, CTL019 1L high risk ALL, pediatrics & young adults	
Entresto[5] LCM sacubitril/valsartan, LCZ696 Post-AMI	**Promacta** LCM eltrombopag, ETB115 Food effect free formulation	**Lutathera** LCM [177]Lu-oxodotreotide[2)] GEP-NET 1L G3	**Tafinlar + Mekinist** LCM dabrafenib + trametinib, DRB436 Thyroid cancer			
Jakavi LCM ruxolitinib, INC424 Chronic GVHD	**Tafinlar + Mekinist** LCM dabrafenib + trametinib, DRB436 HGG/LGG - Pediatrics	**Piqray** LCM alpelisib, BYL719 TNBC	**Tabrecta** LCM capmatinib, INC280 Solid tumors			
Jakavi LCM ruxolitinib, INC424 Acute GVHD	**Xolair** LCM omalizumab, IGE025 Food allergy	**Piqray** LCM alpelisib, BYL719 Ovarian cancer	**Leqvio** LCM KJX839 Ped Hyoerlipidemia			
Kymriah LCM tisagenlecleucel, CTL019 r/r DLBCL 1st relapse	**Xolair** LCM omalizumab, IGE025 Auto-injector	**Promacta** LCM eltrombopag, ETB115 Radiation sickness syndrome				
Kymriah LCM tisagenlecleucel, CTL019 r/r Follicular lymphoma						

1. OAV101 (AVXS-101) IT filing timelines TBC based on HA feedback, preclinical studies to address partial clinical hold are on track 2. Depending on timing of final read-out submission may move to early 2022 3. [177]Lu-dotatate in US
4. Approved in US 5. To be confirmed

References

Tislelizumab	
1	Zhou et al, AACR 2021, Results from RATIONALE 303: Ph3 study of tislelizumab vs. docetaxel as 2L / 3L therapy in locally advanced or metastatic NSCLC. PD-L1 ≥ 25% population included all patients with ≥ 25% of TCs with PD-L1 membrane staining (assessed via Ventana SP263 assay)
2	Descriptive P-value. Data cut-off: August 10th 2020. One-sided P-value was estimated from stratified log-rank test. Hazard ratio was estimated from stratified Cox model with docetaxel group as reference group. Medians were estimated by Kaplan-Meier method with 95% CIs estimated using the method of Brookmeyer and Crowley

Kesimpta®	
1	Based on post-hoc data from the ASCLEPIOS trials Kappos L, Montalban X, Coyle P, et al. Ofatumumab reduces disability progression independent of relapse activity in patients with relapsing multiple sclerosis. ePoster presentation at Virtual AAN Meeting; April 2021
2	For >84.4 kg but representative for all body weights. Modified from Hauser et al., AAN 2020, B-cell Depletion and Efficacy Outcomes with Ofatumumab: Subgroup Analysis from the Pooled Phase 3 ASCLEPIOS I and II Trials; P7.1-013.
3	Cross AH, Delgado S, Habek M, et al. Characteristics and outcome of COVID-19 in patients with relapsing multiple sclerosis receiving ofatumumab. ePoster presentation at Virtual AAN Meeting; April 2021
4	Based on start forms
5	US commercial lives with unrestricted coverage or single step edit
6	SoA leading on virtual and F2F engagement, source: IQVIA BrandImpact report (month ending Feb'21)
7	Source: Symphony Anonymous Patient Level Claims Data (through January 2021)

References

Leqvio®	
	NHS - National Health Service. CV - Cardiovascular
1	World Health Organization. Cardiovascular diseases (CVDs). Available from: https://www.who.int/news-room/fact-sheets/detail/cardiovascular-diseases-(cvds) [Last accessed: September 2020].
2	McClellan M, Brown N, Califf RM, Warner JJ. Call to Action: Urgent Challenges in Cardiovascular Disease: A Presidential Advisory from the American Heart Association. Circulation. 2019;139(9):E44–E54.
3	Decision Resources Group
4	Wong ND, Young D, Zhao Y, et al. Prevalence of the American College of Cardiology/American Heart Association statin eligibility groups, statin use, and low-density lipoprotein cholesterol control in US adults using the National Health and Nutrition Examination Survey 2011–2012. J Clin Lipidol. 2016;10(5):1109–1118
5	Kuiper et al. Use of Lipid-modifying Therapy and LDL-C Goal Attainment in a High-Cardiovascular-Risk Population in the Netherlands. Clin Ther. 2017 Apr;39(4):819-827.e1
6	Fox et al. Treatment patterns and low-density lipoprotein cholesterol (LDL-C) goal attainment among patients receiving high- or moderate-intensity statins. *Clin Res Cardiol*. 2018 May;107(5):380-388
7	Truven claims data, continuously enrolled Jan 2013 – Dec 2017.
8	IQVIA LRx, Xponent data, November 2020
9	Brandts J, et al. Circulation. 2020;141(11):873-876. Grabowski DC, et al. Health Aff. 2012;31(10):2276-2285 Hines DM, et al
10	Fitchett DH, Hegele RA, Verma S. Statin intolerance. *JAMA* 2015;131(13):e389-e391
11	Newman CB, Tobert JA. Statin intolerance – reconciling clinical trials and clinical experience. *JAMA*. 2015;313(10):1011-1012
12	Stroes ES, et al. Statin-associated muscle symptoms: impact on statin therapy-European Atherosclerosis Society Consensus Panel Statement on Assessment, Aetiology and Management. *Eur Heart J*. 2015 May 1;36(17):1012-22
13	Navar AM, et al. PCSK9 Inhibitors: Patient-Reported Barriers to Medication Initiation and Persistence. Circulation. 2017 Nov 14;136(suppl_1):A1912

VISION study	
1	Antonarakis, Emmannel – Current Understanding of Resistance to Abiraterone and Enzalutamide in Advanced Prostate Cancer; Clinical Advances in Hematology & Oncology (2016, Vol. 14, Issue 5)
2	mCRPC pre-taxane study: PSMAfore; mHSPC study: PSMAddition
3	Based on Kantar Health CancerMpact Treatment Architecture US, EU5 and JP (Dec, 2019)

Clinical Trials Update

Includes selected ongoing or recently concluded global trials of Novartis development programs/products which are in confirmatory development or marketed (typically Phase 2b or later).

For further information on all Novartis clinical trials, please visit: www.novartisclinicaltrials.com

Cardiovascular, Renal and Metabolic

Entresto® – Angiotensin II Receptor Neprilysin Inhibitor (ARNI)

Study	NCT02678312 PANORAMA HF (CLCZ696B2319)	NCT03785405 (CLCZ696B2319E1 – extension study)
Indication	Heart failure in pediatric patients	Heart failure in pediatric patients
Phase	Phase 3	Phase 3
Patients	360	240
Primary Outcome Measures	Part 1: Pharmacodynamics and pharmacokinetics of sacubitril/valsartan LCZ696 analytes Part 2: Efficacy and safety compared with enalapril	Number of participants with Adverse Events (AEs) and Serious Adverse Events (SAEs)
Arms/Intervention	• Part 1: Sacubitril/valsartan 0.8 mg/kg or 3.1 mg/kg or both; 0.4 mg/kg or 1.6 mg/kg or both (single doses). • Part 2: enalapril/placebo 0.2 mg/kg bid (ped. formulation 1mg/ml) and adult formulation (2.5, 5, 10 mg bid); Sacubitril/valsartan (LCZ696)/placebo: Ped. formulation granules (12.5, 31.25 mg in capsules); liquid formulation (1mg/ml and 4mg/ml concentration) and adult formulation (50, 100, 200 mg bid)	• Single arm, open label sacubitril/valsartan (pediatric formulation granules (12.5, 31.25 mg in capsules); liquid formulation (1mg/ml and 4mg/ml concentration) and adult formulation (50, 100, 200 mg bid))
Target Patients	Pediatric patients from 1 month to < 18 years of age with heart failure due to systemic left ventricle systolic dysfunction	Pediatric patients with heart failure due to systemic left ventricle systolic dysfunction who have completed study CLCZ696B2319
Read-out Milestone(s)	2022; (Analysis of 110 pts from Part 2 formed the basis for pediatric submission in Apr-2019 and approval by the US FDA in Oct-2019 for the treatment of symptomatic HF with systemic left ventricular systolic dysfunction in children aged 1 year and older)	2023
Publication	TBD	TBD

Entresto® – Angiotensin II Receptor Neprilysin Inhibitor (ARNI)

Study	NCT02884206 PERSPECTIVE (CLCZ696B2320)	NCT02468232 PARALLEL-HF (CLCZ696B1301)
Indication	Heart failure	Heart failure, reduced ejection fraction
Phase	Phase 3	Phase 3
Patients	592	225
Primary Outcome Measures	Change from baseline in the CogState Global Cognitive Composite Score (GCCS)	Time to the first occurrence of the composite endpoint – either cardiovascular (CV) death or heart failure (HF) hospitalization
Arms/Intervention	• Sacubitril/valsartan 50, 100, and 200 mg bid with placebo of valsartan • Valsartan 40, 80, and 160 mg bid tablets with placebo for sacubitril/valsartan	• Sacubitril/valsartan 50 mg, 100 mg, 200 mg bid/placebo of enalapril • Enalapril 2.5 mg, 5 mg, 10 mg bid / placebo of sacubitril/valsartan
Target Patients	Patients with chronic heart failure with preserved ejection fraction	Japanese heart failure patients (NYHA Class II-IV) with reduced ejection fraction
Read-out Milestone(s)	2022	Primary: Q1-2019 *(actual)*; Extension *(open-label)*: H1-2021
Publication	TBD	H1-2021

Entresto® – Angiotensin II Receptor Neprilysin Inhibitor (ARNI)

Study	NCT03066804 PARALLAX (CLCZ696D2302)	NCT02924727 PARADISE-MI (CLCZ696G2301)
Indication	Heart failure, preserved ejection fraction	Post-acute myocardial infarction
Phase	Phase 3	Phase 3
Patients	2,572	5,670
Primary Outcome Measures	Change in NT-proBNP from baseline to week 12 and change in 6 minute walk distance (6MWD) from baseline to Week 24	Time to the first occurrence of a confirmed composite endpoint (cardiovascular (CV) death, heart failure (HF) hospitalization, or outpatient heart failure)
Arms/Intervention	• Sacubitril/valsartan 50 mg, 100 mg and 200 mg bid and matching placebo • Enalapril 2.5 mg, 5 mg and 10 mg bid and matching placebo • Valsartan 40 mg, 80 mg, 160 mg bid and matching placebo	• Sacubitril/valsartan 50 mg, 100 mg, 200 mg bid; placebo for ramipril ; placebo for valsartan • Ramipril 1.25 mg, 2.5 mg, and 5 mg bid; placebo for sacubitril/valsartan; placebo for valsartan
Target Patients	Heart failure patients (NYHA Class II-IV) with preserved ejection fraction	Post-AMI patients with evidence of LV systolic dysfunction and/or pulmonary congestion, with no known prior history of chronic HF
Read-out Milestone(s)	2019 *(actual)*	H1-2021
Publication	• H1-2021	• PARADISE-MI study design / baseline characteristics: publication planned for H1-2021 • Primary data publication planned for H2-2021

KJX839 – siRNA (regulation of LDL-C)

Study	NCT03060577 ORION-3 (CKJX839A12201E1)	NCT03705234 ORION-4 (CKJX839B12301)
Indication	Hypercholesterolemia inc. Atherosclerotic Cardiovascular Disease (ASCVD) and ASCVD risk equivalents Heterozygous Familial Hypercholesterolaemia (HeFH)	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH)
Phase	Phase 2	Phase 3
Patients	490	~15,000
Primary Outcome Measures	LDL-C reduction at Day 210 for Group 1 subjects Changes in other lipids and lipoproteins and reduction of LDL-C of more than 50% for patients that are above LDL-C goal ; longer term exposure and safety.	A composite of major adverse cardiovascular events, defined as: • Coronary heart disease (CHD) death; • Myocardial infarction; • Fatal or non-fatal ischaemic stroke; or • Urgent coronary revascularization procedure
Arms/Intervention	• Group 1 – inclisiran 300mg sc on Day 1 and every 180 days thereafter for up to 4 years. • Group 2- Evolocumab 140mg s.c. injection on Day 1 and every 2 weeks until Day 336, followed by inclisiran 300mg on Day 360, Day 450 and then every 6 months for a planned duration of 4 years.	Arm 1: every 6 month treatment KJX839 300mg (given by subcutaneous injection on the day of randomization, at 3 months and then every 6-months) for a planned median duration of about 5 years Arm 2: matching placebo (given bysubcutaneous injection on the day of randomization, at 3 months and then every 6-months) for a planned median duration of about 5 years.
Target Patients	Patients with HeFH or pre-existing atherosclerotic cardiovascular disease (ASCVD) on background statin +/- ezetimibe therapy	Patient population with mean baseline LDL-C ≥ 100mg/dL
Read-out Milestone(s)	2022	2026
Publication	TBD	TBD

KJX839 – siRNA (regulation of LDL-C)

Study	NCT03851705 ORION-5 (CKJX839A12302)	NCT03814187 ORION-8 (CKJX839A12305B)
Indication	Hypercholesterolemia inc. Homozygous Familial Hypercholesterolemia (HoFH)	Hypercholesterolemia inc. Heterozygous Familial Hypercholesterolaemia (HeFH) and Homozygous Familial Hypercholesterolemia (HoFH)
Phase	Phase 3	Phase 3
Patients	56 randomized 2:1 (inclisiran: placebo)	2,991 entered the study
Primary Outcome Measures	• LDL-C reduction at Day 150 • Changes in PCSK9, other lipids and lipoproteins	• Proportion of subjects achieving prespecified low density lipoprotein cholesterol (LDL-C) targets at end of study • Safety and tolerability profile of long term use of inclisiran
Arms/Intervention	• Part 1: inclisiran 300mg on Day 1 and Day 90 or placebo on Day 1 and Day 90 • Part 2: inclisiran on Day 180 for patients who were randomized to the placebo group only, inclisiran on Day 270 and then every 6 months for a planned duration of 2 years for all patients	Inclisiran 300mg on day 1 (placebo patients entered into study from ORION 9, 10 & 11) or placebo on Day 1 (inclisiran patients entered into study from ORION 9, 10 & 11) then inclisiran 300mg on Day 90 and every 6 months for a planned duation of 3 years
Target Patients	Patients with HoFH with background statin +/- ezetimibe therapy	Patients with HeFH or pre-existing atherosclerotic cardiovascular disease (ASCVD) on background statin +/- ezetimibe therapy and risk equivalents (patients from ORION 9, 10 & 11 studies)
Read-out Milestone(s)	Primary: Q3-2020 *(actual)*; Final: H2-2021	2023
Publication	TBD	TBD

KJX839 – siRNA (regulation of LDL-C) Pediatrics

Study	NCT04659863 ORION-13 (CKJX839C12302)	NCT03814187 ORION-16 (CKJX839A12305B)
Indication	Pediatrics	Pediatrics
Phase	Phase 3	Phase 3
Patients	150	150
Primary Outcome Measures	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to day 330	Percentage (%) change in low-density lipoprotein cholesterol (LDL-C) from baseline to day 330
Arms/Intervention	• Group 1: Inclisiran sodium 300mg on Days 1, 90, 270, placebo on Day 360, inclisiran sodium 300mg on Days 450 and 630; Group 2: Placebo on Days 1, 90, 270, inclisiran sodium 300mg on Days 360, 450 and 630.	• Group 1: Inclisiran sodium 300mg on Days 1, 90, 270, placebo on Day 360, inclisiran sodium 300mg on Days 450 and 630; Group 2: Placebo on Days 1, 90, 270, inclisiran sodium 300mg on Days 360, 450 and 630.
Target Patients	Adolescents (12 to less than 18 years) with homozygous familial hypercholesterolemia (HoFH) and elevated low density lipoprotein cholesterol (LDL-C)	Adolescents (12 to less than 18 years) with heterozygous familial hypercholesterolemia (HeFH) and elevated low density lipoprotein cholesterol (LDL-C)
Read-out Milestone(s)	2023	2023
Publication	TBD	TBD

LNP023 – Factor B inhibition of the complement alternative pathway

Study	NCT03373461 (CLNP023X2203)	NCT04154787 (CLNP023D12201)
Indication	IgA nephropathy (IgAN)	Idiopathic membranous nephropathy (iMN)
Phase	Phase 2	Phase 2
Patients	112	72
Primary Outcome Measures	Change from baseline of log transformed UPCR derived from the 24h urine collections at Baseline and Day 90	Change from baseline of UPCR derived from 24hr urine collections at Baseline and Week 24
Arms/Intervention	• Placebo • LNP023 Dose 1 • LNP023 Dose 2 • LNP023 Dose 3 • LNP023 Dose 4	• LNP023 low dose • LNP023 high dose • Rituximab
Target Patients	Patients with biopsy-verified IgA nephropathy	Patients with biopsy proven iMN who are at high risk of disease progression defined on the basis of antibody anti-PLA2R titre and proteinuria
Read-out Milestone(s)	H1-2021 (IA)	2023
Publication	H1-2021	TBD

LNP023 – Factor B inhibition of the complement alternative pathway

Study	NCT04578834 Applause-IgAN (CLNP023A2301)	NCT04558918 APPLY-PNH (CLNP023C12302)
Indication	IgA nephropathy (IgAN)	Paroxysmal nocturnal hemoglobinuria (PNH)
Phase	Phase 3	Phase 3
Patients	~450	~91
Primary Outcome Measures	• Ratio to baseline in urine protein to creatinine ratio (sampled from 24h urine collection) at 9 months • Annualized total estimated Glomerular Filtration Rate (eGFR) slope estimated over 24 months	• Percentage of participants achieving a sustained increase in hemoglobin levels of ≥ 2 g/dL in the absence of red blood cell transfusions • Percentage of participants achieving sustained hemoglobin levels ≥ 12 g/dL in the absence of red blood cell transfusions
Arms/Intervention	• Arm 1 - LNP023 200mg BID • Arm 2 - Placebo BID	• Arm 1: Drug: LNP023, taken orally b.i.d. dosage supplied: 200 mg dosage (oral) • Arm 2: • Drug: Eculizumab, administered as intravenous infusion every 2 weeks as per the stable regimen, the maintenance dose is a fixed dose (300 mg/30mL) • Drug: Ravulizumab, administered as intravenous infusion every 8 weeks, the maintenance dose is based on body weight (300 mg/30mL)
Target Patients	Primary IgA Nephropathy patients	Adult patients with PNH and residual anemia, despite treatment with an intravenous Anti-C5 antibody
Read-out Milestone(s)	2025	Primary 2022
Publication	TBD	TBD

LNP023 – Factor B inhibition of the complement alternative pathway

Study	NCT03832114 (CLNP023X2202)	NCT03955445 (CLNP023B12001B)
Indication	C3 glomerulopathy (C3G)	C3 glomerulopathy (C3G)
Phase	Phase 2	Phase 2 (open-label extension)
Patients	27	27 patients from ongoing Ph2 (sample size from Ph3 pending HA discussions Q1 2021), total patients for this study will increase
Primary Outcome Measures	Cohort A: Ratio to Baseline of UPCR to Week 12 derived from 24hr urine collection Cohort B: Change from Baseline in C3 Deposit Score (based on immunofluorescence microscopy) at Week 12	Characterize the effect of LNP023 treatment on a composite renal response endpoint at 9 months (1. a stable or improved eGFR and, 2. a reduction in proteinuria and 3. an increase in C3 compared to the CLNP023X2202 baseline visit)
Arms/Intervention	Increasing doses of LNP023 up to 200mg bid: • Cohort A: Native kidney patients • Cohort B: Kidney transplanted patients	• Open-label LNP023 200mg bid
Target Patients	Patients with C3 glomerulopathy	Patients with C3 glomerulopathy
Read-out Milestone(s)	H1-2021	2025
Publication	Interim analysis data from Cohort-A presented at American Society of Nephrology (ASN 2020)	H2-2021

LNP023 – Factor B inhibition of the complement alternative pathway

Study	NCT03439839 (CLNP023X2201)	NCT03896152 (CLNP023X2204)
Indication	Paroxysmal nocturnal hemoglobinuria (PNH)	Paroxysmal nocturnal hemoglobinuria (PNH)
Phase	Phase 2	Phase 2
Patients	16	13
Primary Outcome Measures	Reduction of chronic hemolysis, based on LDH level at Week 13	Reduction of PNH associated hemolysis, based on percentage of patients with 60% reduction in LDH or LDH below upper limit of normal up to 12 weeks of treatment.
Arms/Intervention	• 10 patients receiving LNP023 high dose daily over up to approximately 3 years • 5 patients receiving LNP023 low dose daily over up to approximately 3 years	• approximately 2 year Treatment with low LNP023 dose • approximately 2 year Treatment with higher LNP023 dose
Target Patients	Patients with PNH, showing signs of active hemolysis despite treatment with SoC (defined as an antibody with anti C5 activity).	Patients with PNH, showing signs of active hemolysis, not treated with any other complement inhibitor less than 3 months prior to study start Day 1
Read-out Milestone(s)	Primary: Q2-2020 *(actual)* Extension: 2023	Primary: Q2-2020 *(actual)* Extension: 2022
Publication	Antonio M. Risitano, MD, PhD1 et al. Presented at EBMT 2020 congress Lancet Haematol - Study of Safety, Efficacy, Tolerability, Pharmacokinetics and Pharmacodynamics of LNP023 in in Patients With Paroxysmal Nocturnal Hemoglobinuria (PNH)	H1-2021

LNP023 – Factor B inhibition of the complement alternative pathway

Study	NCT04820530 APPOINT-PNH (CLNP023C12301)
Indication	Paroxysmal nocturnal hemoglobinuria (PNH)
Phase	Phase 3
Patients	~40
Primary Outcome Measures	• Proportion of participants achieving a sustained increase from baseline in hemoglobin levels of ≥ 2 g/dL assessed , in the absence of red blood cell transfusions
Arms/Intervention	Iptacopan (LNP023), taken orally b.i.d. (dosage supplied: 200mg)
Target Patients	PNH patients who are naive to complement inhibitor therapy, including anti-C5 antibody
Read-out Milestone(s)	2023
Publication	TBD

TQJ230 – Antisense oligonucleotide targeting apolipoprotein(a) mRNA

Study	NCT04023552 Lp(a)HORIZON (CTQJ230A12301)
Indication	Cardiovascular risk reduction
Phase	Phase 3
Patients	7,680
Primary Outcome Measures	Time to the first occurrence of MACE (cardiovascular death, non-fatal MI, non-fatal stroke and urgent coronary re-vascularization)
Arms/Intervention	TQJ230 80 mg injected monthly subcutaneously or matched placebo
Target Patients	Patients with a history of Myocardial infarction or Ischemic Stroke, or a clinically significant symptomatic Peripheral Artery Disease, and Lp(a) ≥ 70 mg/dL
Read-out Milestone(s)	2024
Publication	TBD

Immunology, Hepatology & Dermatology

CFZ533 – Blocking, non-depleting, Fc-silent, anti-CD40 monoclonal antibody

Study	NCT03663335 CIRRUS I (CCFZ533A2201)	NCT03905525 TWINSS (CCFZ533B2201)
Indication	Kidney transplantation	Sjögren's syndrome
Phase	Phase 2	Phase 2
Patients	681	260
Primary Outcome Measures	Cohorts 1 and 2-mean iBox risk prediction score at 12 months. Integrative score that will provide a prediction of graft survival at year 5	Change in EULAR Sjögren's syndrome Disease Activity Index (ESSDAI) score and EULAR Sjögren's syndrome Patient Reported Index (ESSPRI) score
Arms/Intervention	• Two cohorts: de novo TX and maintenance • Test Arms: CFZ533 + MMF + corticosteroids • Standard of Care: TAC + MMF + corticosteroids	• Three dose arms of CFZ533 • Placebo
Target Patients	Kidney transplant recipients	Patients with Sjögren's syndrome
Read-out Milestone(s)	2022	2022
Publication	2022	2022

CFZ533 – Blocking, non-depleting, Fc-silent, anti-CD40 monoclonal antibody

Study	NCT03781414 CONTRAIL I (CCFZ533A2202)
Indication	Liver transplantation
Phase	Phase 2
Patients	128
Primary Outcome Measures	Proportion of patients with composite event (BPAR, Graft Loss or Death) over 12 months
Arms/Intervention	• Control/Standard of Care: TAC + MMF + Corticosteroids • CFZ533 dose A + MMF + Corticosteroids • CFZ533 dose B + MMF + Corticosteroids
Target Patients	Liver transplant recipients
Read-out Milestone(s)	2023
Publication	2023

Cosentyx® – Anti IL-17

Study	NCT03504852 (CAIN457A2324)	NCT03589885 MATURE (CAIN457A2325)
Indication	Psoriasis	Psoriasis
Phase	Phase 3B	Phase 3
Patients	331	122
Primary Outcome Measures	PASI 90 response and IGA mod 2011 0 or 1 response after 16 weeks of treatment	PASI 75 response and IGA mod 2011 0 or 1 response after 12 weeks of treatment
Arms/Intervention	• Secukinumab 300 mg every 2 weeks after weekly doses till Week 4 • Secukinumab 300 mg every 4 weeks after weekly doses till Week 4	• Secukinumab 2 mL (300 mg) auto-injector • Secukinumab 2 x 1 mL (150 mg each) prefilled syringe • Placebo 2 mL auto-injector • Placebo 2 x 1 mL prefilled syringe
Target Patients	Subjects (≥90kg) with moderate to severe plaque psoriasis	Subjects with moderate to severe plaque psoriasis
Read-out Milestone(s)	Q3-2020 *(actual)*	Final: Q4-2020 *(actual)*
Publication	Publication (primary efficacy) planned in H1-2021	16-week results AAD 2021 52-week results H1-2021

Cosentyx® – Anti IL-17

Study	NCT02471144 (CAIN457A2310)	NCT03668613 (CAIN457A2311)
Indication	Psoriasis	Psoriasis
Phase	Phase 3	Phase 3
Patients	162	84
Primary Outcome Measures	Psoriasis Area and Severity Index (PASI) 75 response and Investigators' Global Assessment (IGA) 0 or 1 response at week 12	Psoriasis Area and Severity Index (PASI) 75 response and Investigators' Global Assessment (IGA) 0 or 1 response at week 12
Arms/Intervention	• Secukinumab low dose • Secukinumab high dose • Placebo • Etanercept (comparator)	• Secukinumab low dose • Secukinumab high dose
Target Patients	Patients from 6 to less than 18 years of age with severe chronic plaque psoriasis	Pediatric patients of age 6 to <18 years, with moderate to severe plaque psoriasis
Read-out Milestone(s)	2023	2023
Publication	Published Q4 2020 JEADV Further congress plans in 2021	H1-2021

Cosentyx® – Anti IL-17

Study	NCT03066609 (CAIN457A2318)
Indication	Psoriasis
Phase	Phase 3
Patients	543
Primary Outcome Measures	Psoriasis Area and Severity Index (PASI) 75 response and Investigators' Global Assessment (IGA) 0 or 1 response at week 12
Arms/Intervention	• Secukinumab 300 mg • Secukinumab 150 mg • Placebo
Target Patients	Patients with moderate to severe chronic plaque-type psoriasis with or without psoriatic arthritis comorbidity
Read-out Milestone(s)	Q1-2019 *(actual)*
Publication	• Week 16 results: Poster presented at: 2019 American Academy of Dermatology (AAD) Annual Meeting, • March 1–5, 2019, Washington, D.C. • 52-week results: Poster at EADV 2019, Madrid 9-13 October, 2019 • Manuscript publication H1-2021

Cosentyx® – Anti IL-17

Study	NCT03031782 (CAIN457F2304)	NCT03769168 (CAIN457F2304E1 – extension study)
Indication	Psoriatic arthritis	Psoriatic arthritis
Phase	Phase 3	Phase 3
Patients	80	64
Primary Outcome Measures	Time to 33 flares	Number of participants with JIA ACR30 response
Arms/Intervention	• Secukinumab (pre-filled syringe) 75 mg • Placebo	• Secukinumab 75 mg/0.5 ml • Secukinumab 150 mg/1.0 ml
Target Patients	Juvenile idiopathic arthritis subtypes of psoriatic and enthesitis-related arthritis	Patients with juvenile idiopathic arthritis subtypes of juvenile psoriatic arthritis and enthesitis related arthritis
Read-out Milestone(s)	H1-2021 (actual)	2025
Publication	H2-2021	TBD

Cosentyx® – Anti IL-17

Study	NCT03259074 SURPASS (CAIN457K2340)	NCT03713632 SUNRISE (CAIN457M2302)
Indication	Ankylosing spondylitis	Hidradenitis Suppurativa (HS)
Phase	Phase 3	Phase 3
Patients	837	471
Primary Outcome Measures	No radiographic structural progression as measured by modified Stoke Ankylosing Spondylitis Spine Score (mSASSS)	Proportion of patients with Hidradenitis Suppurativa Clinical Response (HiSCR)
Arms/Intervention	• Secukinumab 150/300 mg • Adalimumab biosimilar 40 mg	• Secukinumab 300 mg every 2 weeks • Secukinumab 300 mg every 4 weeks • Placebo (every 2 weeks) • Placebo (every 4 weeks)
Target Patients	Patients with active ankylosing spondylitis	Subjects with moderate to severe Hidradenitis Suppurativa
Read-out Milestone(s)	2022	Primary (week 16): H2-2021; Final: 2022
Publication	• Study design manuscript published. Baraliakos et al. Clinical Drug Investigation (2020) 40:269–278.	StudStudy design SHSA 2020; Primary 2022

Cosentyx® – Anti IL-17

Study	NCT03713619 SUNSHINE (CAIN457M2301)	NCT04179175 (CAIN457M2301E1)
Indication	Hidradenitis Suppurativa (HS)	Hidradenitis Suppurativa (HS)
Phase	Phase 3	Phase 3
Patients	471	745
Primary Outcome Measures	Proportion of participants with Hidradenitis Suppurativa clinical response (HiSCR)	Proportion of patients with Hidradenitis Suppurativa Clinical Response (HiSCR)
Arms/Intervention	• Secukinumab 300 mg every 2 weeks • Secukinumab 300 mg every 4 weeks • Placebo (every 2 weeks) • Placebo (every 4 weeks)	• Secukinumab 300 mg every 2 weeks • Secukinumab 300 mg every 4 weeks
Target Patients	Patients with moderate to severe Hidradenitis Suppurativa	Patients with moderate to severe hidradenitis suppurativa completing either of the core trials AIN457M2301 (NCT 0313632) or AIN567M2302 (NCT03713619)
Read-out Milestone(s)	Primary (week 16): H2-2021; Final: 2022	2025
Publication	Study design SHSA 2020; Primary 2022	Study design SHSA 2020

Cosentyx® – Anti IL-17

Study	NCT04156620 INVIGORATE-1 (CAIN457P12301)	NCT04209205 INVIGORATE-2 (CAIN457P12302)
Indication	Axial spondyloarthritis	Psoriatic Arthritis (PsA)
Phase	Phase 3	Phase 3
Patients	500	380
Primary Outcome Measures	The proportion of subjects achieving an ASAS40 (Assessment of SpondyloArthritis International Society criteria) response	The proportion of subjects achieving American College of Rheumatology 50 (ACR50) response criteria
Arms/Intervention	• Secukinumab intravenous (i.v.) regimen • Placebo intravenous (i.v.) regimen	• Secukinumab intravenous (i.v.) regimen • Placebo intravenous (i.v.) regimen
Target Patients	Patients with active axial spondyloarthritis	Patients with active psoriatic arthritis (PsA) despite current or previous NSAID, DMARD and/or anti-TNF therapy
Read-out Milestone(s)	2023	2022
Publication	TBD	2023

Cosentyx® – Anti IL-17

Study	NCT04181762 SELUNE (CAIN457Q12301)	NCT04300296 PRELUDE (CAIN457S12201)
Indication	Lupus Nephritis	Lichen Planus
Phase	Phase 3	Phase 2
Patients	460	108
Primary Outcome Measures	Proportion of subjects achieving protocol-defined CRR	Proportion of patients achieving Investigator's Global Assessment (IGA 0/1) score at 16 weeks +30% delta vs placebo
Arms/Intervention	• Secukinumab 300 mg s.c. • Placebo s.c.	• Secukinumab 300 mg s.c. • Placebo s.c.
Target Patients	Patients with active lupus nephritis (ISN/RPS Class III or IV, with or without co-existing class V features)	Adult patients with biopsy-proven lichen planus not adequately controlled by topical therapies
Read-out Milestone(s)	2026	2022
Publication	**2026**	TBD

LJC242 – FXR agonist + CCR2/CCR5 inhibitor

Study	NCT03517540 TANDEM (CLJC242A2201J)
Indication	Non-alcoholic steatohepatitis
Phase	Phase 2
Patients	193
Primary Outcome Measures	Evaluation of safety and tolerability of combination therapy (tropifexor + cenicriviroc) by monitoring adverse event profile, vital signs and laboratory parameters
Arms/Intervention	• Arm A: tropifexor (LJN452) dose 1 • Arm B: cenicriviroc (CVC) • Arm C: LJN452 dose 1 + CVC • Arm D: LJN452 dose 2 + CVC
Target Patients	Adult patients with non-alcoholic steatohepatitis (NASH) and liver fibrosis
Read-out Milestone(s)	Q4-2020 (actual)
Publication	Abstract planned in H1-2021

LJN452 – FXR Agonist

Study	NCT04065841 ELIVATE (CLJN452D12201C)
Indication	Non-alcoholic steatohepatitis (NASH)
Phase	Phase 2
Patients	380
Primary Outcome Measures	Proportion of patients with resolution of NASH and no worsening of fibrosis OR improvement in fibrosis by at least one stage without worsening of NASH at Week 48 compared with baseline
Arms/Intervention	• Arm A: combination therapy tropifexor + licogliflozin • Arm B: tropifexor monotherapytropifexor + licogliflozin placebo • Arm C: licogliflozin monotherapylicogliflozin + tropifexor placebo • Arm D: licogliflozin placebo + tropifexor placebo
Target Patients	Adult patients with biopsy based non-alcoholic steatohepatitis (NASH) and liver fibrosis
Read-out Milestone(s)	2022
Publication	Planned in H1-2023

LNA043 – ANGPTL3 Agonist

Study	NCT03275064 (CLNA043X2202)
Indication	Knee Osteoarthritis
Phase	Phase 2
Patients	~133
Primary Outcome Measures	• Articular cartilage bi-layer collagen organisation evaluated with MRI and measured in milliseconds (ms) (Part A only) • Number of patients with any adverse events, serious adverse events and death (Part A and Part B) • Change in cartilage volume/thickness in the index region (Part B only)
Arms/Intervention	• LNA043 40 mg Part B • LNA043 20 mg Part B • LNA043 20 mg Part A • Placebo Part A • Placebo Part B
Target Patients	Patients with cartilage lesions of the knee (Part A) and knee osteoarthritis (Part B)
Read-out Milestone(s)	2022
Publication	TBD

LOU064 – Bruton's tyrosine kinase (BTK) inhibitor

Study	NCT03926611 (CLOU064A2201)	NCT04109313 (CLOU064A2201E1)
Indication	Chronic spontaneous urticaria (CSU)	Chronic spontaneous urticaria (CSU)
Phase	Phase 2	Phase 2
Patients	308	250
Primary Outcome Measures	Change from baseline in weekly Urticaria Activity Score (UAS7) at Week 4	• Long-term safety and tolerability
Arms/Intervention	• Arm 1 Low dose of LOU064 orally in the morning (once daily) and matching placebo in the evening from Day 1 to 85 • Arm 2 Medium dose of LOU064 orally in the morning (once daily) and matching placebo in the evening from Day 1 to 85 • Arm 3 High dose of LOU064 orally in the morning (once daily) and matching placebo in the evening from Day 1 to 85 • Arm 4 Low dose of LOU064 orally, twice daily from Day 1 to 85 • Arm 5 Medium dose of LOU064 orally, twice daily from Day 1 to 85 • Arm 6 High dose of LOU064 orally, twice daily from Day 1 to 85 • Placebo arm Matching placebo, orally, twice daily from Day 1 to 85	• Selected dose of LOU064 taken orally twice a day (morning and evening) from day 1 to week 52
Target Patients	Adults with CSU inadequately controlled by H1-antihistamines	Patients with CSU who have participated in preceding studies with LOU064
Read-out Milestone(s)	H2-2021	2022
Publication	H2-2021	TBD

QGE031 – Anti-IgE

Study	NCT03437278 (CQGE031C2202)	NCT04210843 (CQGE031C2302E1)
Indication	Chronic spontaneous urticaria	Chronic spontaneous urticaria
Phase	Phase 2	Phase 3
Patients	48	800
Primary Outcome Measures	Change in the 7 day Urticaria Activity Score (UAS7)	The proportion of subjects with well-controlled disease (UAS7 ≤ 6) at week 12
Arms/Intervention	• Ligelizumab high dose q4wks for 24 weeks • Ligelizumab low dose q4wks for 24 weeks • Placebo / ligelizumab high dose q4wks for 8 / 16 weeks	• Ligelizumab Dose 1 and 3 • Ligelizumab Dose 2 and 3
Target Patients	Adolescents from 12 to <18 years of age, with chronic spontaneous urticaria	Patients who completed studies CQGE031C2302, CQGE031C2303, CQGE031C2202 or CQGE031C1301
Read-out Milestone(s)	H2-2021	2026
Publication	• Study design was presented at PAAM (Peds Allergy & Asthma Meeting) and at UCARE meeting 2019 • Baseline characteristics 2020/21 • Primary results to be presented in late 2021/2022 (e.g. EAACI, PAAM, EADV) • Manuscript to be submitted in 2022	Study design presented at 2020 EAACI

QGE031 – Anti-IgE

Study	**NCT02649218 (CQGE031C2201E1)**
Indication	Chronic spontaneous urticaria
Phase	Phase 2
Patients	226
Primary Outcome Measures	Long-term safety; number of participants with treatment-emergent adverse events
Arms/Intervention	Ligelizumab 240 mg q4wks open label for 52 weeks
Target Patients	Adult patients with chronic spontaneous urticaria inadequately controlled with H_1-antihistamines at approved or increased doses, alone or in combination with H_2-antihistamines or leukotriene receptor antagonists.
Read-out Milestone(s)	2019 *(actual)*
Publication	• H1-2021 manuscript: primary results extension trial (NEJM) • 2021 Congresses: exploratory data AAAAI, AAD, EAACI, EADV, ACAAI, encores at GUF

QGE031 – Anti-IgE

Study	NCT03580369 Pearl 1 (CQGE031C2302)	NCT03580356 Pearl 2 (CQGE031C2303)
Indication	Chronic spontaneous urticarial	Chronic spontaneous urticarial
Phase	Phase 3	Phase 3
Patients	1,050	1,050
Primary Outcome Measures	Absolute change from baseline in UAS7 (Urticaria Activity Score) at week 12	Absolute change from baseline in UAS7 (Urticaria Activity Score) at week 12
Arms/Intervention	• Ligelizumab dose A q4w for 52 weeks • Ligelizumab dose B q4w for 52 weeks • Omalizumab 300 mg q4w for 52 weeks • Placebo q4w from randomization to wk20, then ligelizumab dose B from wk24 to wk52	• Ligelizumab dose A q4w for 52 weeks • Ligelizumab dose B q4w for 52 weeks • Omalizumab 300 mg q4w for 52 weeks • Placebo q4w from randomization to wk20, then ligelizumab dose B from wk24 to wk52
Target Patients	Adolescents and adults with chronic spontaneous urticaria inadequately controlled with H1-antihistamines	Adolescents and adults with chronic spontaneous urticaria inadequately controlled with H1-antihistamines
Read-out Milestone(s)	H2-2021 (Q4/2021-Q1/2022 potential COVID impact)	H2-2021 (Q4/2021-Q1/2022 potential COVID impact)
Publication	• Study design presented at UCARE 2018 • Primary results to be presented in 2022 (e.g. EAACI, PAAM, EADV) • Manuscript to be submitted in 2022	

VAY736 – Fully human IgG1/κ anti-BAFF-R mAb

Study	NCT03217422 AMBER (CVAY736B2201)
Indication	Autoimmune hepatitis
Phase	Phase 2
Patients	80
Primary Outcome Measures	Alanine aminotransferase (ALT) normalization
Arms/Intervention	• VAY736 • Placebo control with conversion to active VAY736
Target Patients	Autoimmune hepatitis patients with incomplete response or intolerant to standard treatment of care
Read-out Milestone(s)	2026
Publication	TBD

Neuroscience

Aimovig® – CGRP receptor antagonist

Study	NCT03096834 LIBERTY (CAMG334A2301)	NCT03333109 EMPOWER (CAMG334A2302)
Indication	Migraine	Migraine
Phase	Phase 3	Phase 3
Patients	246	900
Primary Outcome Measures	Percentage of patients with a 50% response in the reduction of Monthly Migraine Days (MMD)	Change from baseline in monthly migraine days at the last month (Month 3) of the double-blind treatment period
Arms/Intervention	• Subcutaneous injection of AMG334 (erenumab) • Subcutaneous injection of placebo	• AMG334 (erenumab) Dose 1 • AMG334 (erenumab) Dose 2 • Placebo
Target Patients	Adult episodic migraine patients who have failed prophylactic migraine treatments	Adult episodic migraine patients
Read-out Milestone(s)	Double-blind: 2017 *(actual)*; Extension (open-label): H1-2021	Q1-2020 *(actual)*
Publication	• PROs and prespecified subgroup analysis (Double-blind phase) submitted to JNNP accepted Aug-2020 • Submitted May 28, 2020 1 year Open-label extension to Neurology • Planned for Q4-2020: 2Y Open-label extension Abstracts completed for EAN, AHS, EHF and MTIS in 2020	• Primary analysis manuscript submitted end 2020 • Abstracts accepted for MTIS in 2020 • Secondary analysis to be submitted to multiple congresses in 2021

Aimovig® – CGRP receptor antagonist

Study	NCT03867201 DRAGON (CAMG334A2304)
Indication	Migraine
Phase	Phase 3
Patients	550
Primary Outcome Measures	Change from baseline in monthly migraine days during the last 4 weeks of the 12-week treatment period
Arms/Intervention	• Subcutaneous injection of AMG334 (erenumab) 70 mg • Subcutaneous injection of placebo
Target Patients	Adult chronic migraine patients
Read-out Milestone(s)	Double-blind:2021; Extension (open-label): 2024
Publication	Planned in H2-2022 for double-blind phase and H1-2025 for open-label extension phase

LMI070 – SMN2 RNA splice modulator

Study	NCT02268552 (CLMI070X2201)
Indication	Type 1 spinal muscular atrophy
Phase	Phase 1/2
Patients	39
Primary Outcome Measures	Number of participants with adverse events (AEs), serious adverse events (SAEs) and deaths
Arms/Intervention	Branaplam oral, once weekly: • Part 1: 5 ascending doses • Part 2: 2 different dose levels • Part 3: patients continue on initial dose assigned in Part 1 or Part 2
Target Patients	Patients with type 1 spinal muscular atrophy
Read-out Milestone(s)	Study Part 2: Q3-2020 *(actual)* Study Part 3: 2023
Publication	TBD

OMB157 – Anti-CD20

Study	NCT03249714 APOLITOS (COMB157G1301)	NCT03650114 ALITHIOS (COMB157G2399)
Indication	Multiple sclerosis	Multiple Sclerosis
Phase	Phase 2	Phase 3
Patients	60	2010
Primary Outcome Measures	Reduced cumulative number of Gd-enhanced T1 lesions across 4 MRI scans at week 12, 16, 20 and 24 (ofatumumab vs placebo)	Evaluate the long-term safety and tolerability of ofatumumab 20 mg subcutaneous (sc) once every 4 (q4) weeks in subjects with RMS from the first dose of ofatumumab
Arms/Intervention	• Ofatumumab 20 mg subcutaneous injections • Placebo	• Ofatumumab 20 mg every 4 weeks
Target Patients	Patients with relapsing forms of multiple sclerosis	Patients with relapsing MS
Read-out Milestone(s)	Q1-2020 *(actual)*	2028
Publication	Publication planned for H1-2021	TBD

Zolgensma® – SMN1 gene replacement therapy

Study	NCT03505099 SPR1NT (CL-304)	NCT03837184 STR1VE Asia Pacific (CL-306)
Indication	Spinal muscular atrophy	Type 1 spinal muscular atrophy
Phase	Phase 3	Phase 3
Patients	30	2
Primary Outcome Measures	• [2 copies of SMN2] Percentage of participants achieving functional independent sitting for at least 30 seconds at any visit • [3 copies of SMN2] Percentage of participants achieving the ability to stand without support for at least 3 seconds at any visit	Proportion of participants sitting without support
Arms/Intervention	Open-label, single-arm, single-dose, intravenous	Open-label, single-arm, single-dose, intravenous
Target Patients	Pre-symptomatic patients with spinal muscular atrophy and multiple copies SMN2	Patients with spinal muscular atrophy Type 1
Read-out Milestone(s)	H2-2021	H2-2021
Publication	(Muscular Dystrophy Association) MDA 2021 (March 15–18) and (American Academy of Neurology) AAN 2021 (April 17–22)	TBD

Zolgensma® – SMN1 gene replacement therapy

Study	NCT03381729 STRONG (CL-102)
Indication	Type 2 spinal muscular atrophy
Phase	Phase 1
Patients	51
Primary Outcome Measures	• Safety and tolerability, incidence of adverse events • Proportion of patients achieving Standing Milestone • Change in Hammersmith Functional Motor Scale
Arms/Intervention	Open-label, single-arm, single-dose, intrathecal
Target Patients	Patients with spinal muscular atrophy with 3 copies of SMN2
Read-out Milestone(s)	Cohort B: Q4-2019 *(actual);* Cohort C[1]: TBC
Publication	TBD

[1] FDA placed a partial hold on AVXS-101 intrathecal clinical trials for SMA patients based on findings in a small pre-clinical animal study

Oncology

ABL001 – Specific, allosteric Bcr-Abl kinase inhibitor

Study	NCT03106779 ASCEMBL (CABL001A2301)
Indication	Chronic myeloid leukaemia (CML)
Phase	Phase 3
Patients	233
Primary Outcome Measures	Major Molecular Response (MMR) rate at 24 weeks
Arms/Intervention	• ABL001 40 mg bid • Bosutinib 500 mg
Target Patients	Patients with chronic myelogenous leukemia in chronic phase, previously treated with 2 or more tyrosine kinase inhibitors
Read-out Milestone(s)	Q3-2020 *(actual)*
Publication	• Hochhaus A., et al. [Efficacy and Safety Results from ASCEMBL, a Multicenter, Open-Label, Phase 3 Study of Asciminib, a First-in-Class STAMP Inhibitor, vs Bosutinib (BOS) in Patients (Pts) with Chronic Myeloid Leukemia in Chronic Phase (CML-CP) Previously Treated with ≥2 Tyrosine Kinase Inhibitors (TKIs), LBA-4] ASH 2020 • Manuscript submission H1-2021

ACZ885 – IL-1β inhibitor

Study	NCT03447769 CANOPY-A (CACZ885T2301)	NCT03631199 CANOPY-1 (CACZ885U2301)
Indication	Adjuvant NSCLC	1st Line Non-small cell lung cancer (NSCLC)
Phase	Phase 3	Phase 3
Patients	1,500	627
Primary Outcome Measures	Disease free survival (primary), overall survival (key secondary)	• Safety run-in part: Incidence of dose limiting toxicities • Double-blind, randomized, placebo-controlled part: Progression free survival (PFS) • Overall survival (OS)
Arms/Intervention	• Canakinumab 200mg q3w sc for 18 cycles • Placebo q3w sc for 18 cycles	• Canakinumab or matching placebo in combination with pembrolizumab and platinum-based doublet chemotherapy
Target Patients	Patients with: • High–risk NSCLC (AJCC/UICC v.8 stage II-IIIA and IIIB (T>5cm N2)) after complete resection and standard of care adjuvant cisplatin-based chemotherapy • All histologies	Patients with • Histologically confirmed Stage IIIB, IV NSCLC with no prior systemic anticancer therapy • Squamous and non-squamous NSCLC • No EGFR mutation and ALK rearrangement
Read-out Milestone(s)	2023	H2-2021
Publication	TBD	• Johnson B et al. Presented at AACR-NCI-EORTC 2019 (safety run-in) • Planned abstract submission to congress in 2H 2021

ACZ885 – IL-1β inhibitor

Study	NCT03626545 CANOPY-2 (CACZ885V2301)
Indication	2nd / 3rd Line Non-small cell lung cancer (NSCLC)
Phase	Phase 3
Patients	240
Primary Outcome Measures	• Safety run-in part: Incidence of dose limiting toxicities • Double-blind, randomized, placebo-controlled part: Overall Survival
Arms/Intervention	• Canakinumab in combination with docetaxel • Canakinumab matching-placebo in combination with docetaxel
Target Patients	Patients with: • Stage IIIB or IV NSCLC without EGFR, ALK, ROS-1 or B-RAF mutation • Previously treated with platinum therapy and PD(L)1-inhibitor
Read-out Milestone(s)	H1-2021
Publication	Planned abstract submission to congress in 2H 2021

BYL719 – Alpha-specific PI3K inhibitor

Study	NCT04208178 EPIK-B2 (CBYL719G12301)	NCT04251533 EPIK-B3 (CBYL719H12301)
Indication	HER-2 positive breast cancer	Triple negative breast cancer
Phase	Phase 3	Phase 3
Patients	548	566
Primary Outcome Measures	Progression-free survival (PFS)	Progression-free Survival (PFS) for patients with PIK3CA mutant status
Arms/Intervention	• Alpelisib + trastuzumab + pertuzumab • Trastuzumab + pertuzumab	• Alpelisib 300 mg + nab-paclitaxel 100 mg/m² • Placebo + nab-paclitaxel 100 mg/m²
Target Patients	Patients with HER2-positive advanced breast cancer with a PIK3CA mutation	Patients with advanced triple negative breast cancer with either Phosphoinositide-3-kinase Catalytic Subunit Alpha (PIK3CA) mutation or Phosphatase and Tensin Homolog Protein (PTEN) loss without PIK3CA mutation
Read-out Milestone(s)	2025	2023
Publication	TBD	TBD

INC280 – MET Inhibitor

Study	NCT04427072 (CINC280A2301)
Indication	Non-small cell lung cancer
Phase	Phase 2
Patients	90
Primary Outcome Measures	Progression free survival (PFS) per blinded independent review committee (BIRC) using RECIST v1.1
Arms/Intervention	• Arm 1: 400mg of capmatinib tablets administered orally twice daily • Arm 2: Docetaxel 75 mg/m2 by intravenous infusion every 21 days
Target Patients	Previously Treated Patients With EGFR wt, ALK Negative, Locally Advanced or Metastatic (Stage IIIB/IIIC or IV) NSCLC Harboring MET Exon 14 Skipping Mutation (METΔex14).
Read-out Milestone(s)	Primary 2022 Final: 2024
Publication	TBD

Jakavi® – JAK1/2 inhibitor

Study	NCT03112603 REACH3 (CINC424D2301)	NCT03491215 REACH4 (CINC424F12201)
Indication	Steroid-refractory chronic graft vs. host disease (SR cGVHD)	Acute graft versus host disease
Phase	Phase 3	Phase 2
Patients	330	45
Primary Outcome Measures	Overall Response Rate (ORR) at 183 Days	• Measurement of PK parameters • Overall Response Rate (ORR)
Arms/Intervention	• Ruxolitinib 10mg bid • Best available therapy (BAT)	• Ruxolitinib
Target Patients	Patients with SR cGVHD	Pediatric patients with grade II-IV acute graft vs. host disease after allogeneic hematopoietic stem cell transplantation
Read-out Milestone(s)	Final: Q3-2020 *(actual)*	2023
Publication	• Planned manuscript submission in H1-2021 • REACH3 primary analysis oral presentation at ASH (American Society of Hematology) 2020	TBD

Jakavi® – JAK1/2 inhibitor

Study	NCT03774082 REACH5 (CINC424G12201)	NCT04097821 ADORE (CINC424H12201)
Indication	Chronic graft versus host disease	Myelofibrosis
Phase	Phase 2	Phase 1/2
Patients	42	130
Primary Outcome Measures	• Overall Response Rate (ORR)	• Incidence of dose limiting toxicities within the first 2 cycles • Response rate at the end of cycle 6
Arms/Intervention	• Ruxolitinib 5mg tablets / pediatric formulation	• Ruxolitinib • Ruxolitinib+Siremadlin Ruxolitinib+Crizanlizumab • Ruxolitinib+MBG453 • Ruxolitinib+LTT462 • Ruxolitinib+NIS793
Target Patients	Pediatric subjects with moderate and severe chronic Graft vs. Host disease after allogeneic stem cell transplantation	Patients with Myelofibrosis (MF)
Read-out Milestone(s)	2023	2024
Publication	TBD	TBD

Kisqali® – CDK 4/6 inhibitor

Study	NCT03701334 NATALEE (CLEE011O12301C)
Indication	Adjuvant treatment of hormone receptor (HR)-positive, HER2-negative, early breast cancer (EBC)
Phase	Phase 3
Patients	~5,000
Primary Outcome Measures	Invasive Disease-Free Survival for using STEEP criteria (Standardized Definitions for Efficacy End Points in adjuvant breast cancer trials)
Arms/Intervention	• Ribociclib + endocrine therapy • Endocrine therapy
Target Patients	Pre and postmenopausal women and men with HR-positive, HER2-negative EBC, after adequate surgical resection, who are eligible for adjuvant endocrine therapy
Read-out Milestone(s)	2022
Publication	TBD

Kymriah® – CAR-T therapy

Study	NCT03568461 ELARA (CCTL019E2202)	NCT03876769 CASSIOPEIA (CCTL019G2201J)
Indication	Relapsed / refractory follicular lymphoma (FL)	1st line high risk acute lymphoblastic leukemia (ALL)
Phase	Phase 2	Phase 2
Patients	97	160
Primary Outcome Measures	Complete Response Rate (CRR)	Disease Free Survival (DFS)
Arms/Intervention	Single-arm study of tisagenlecleucel	Single-arm study of tisagenlecleucel
Target Patients	Adult patients with relapsed or refractory FL	Pediatric and young adult patients with 1st line high risk ALL
Read-out Milestone(s)	H1-2021	2025
Publication	Planned abstract submission to congress in H2-2021	TBD

Kymriah® – CAR-T therapy

Study	NCT03570892 BELINDA (CCTL019H2301)
Indication	2nd line Diffuse large B-cell lymphoma (DLBCL)
Phase	Phase 3
Patients	318
Primary Outcome Measures	Event-free Survival (EFS)
Arms/Intervention	Tisagenlecleucel versus standard of care
Target Patients	Adult patients with aggressive B-cell Non-Hodgkin Lymphoma after failure of rituximab and anthracycline- containing frontline immunochemotherapy
Read-out Milestone(s)	H2-2021
Publication	• Bishop et al at SITC 2019 • Abstract submission to congress in H2-2021

MBG453 – TIM-3 antagonist

Study	NCT03946670 STIMULUS MDS-1 (CMBG453B12201)	NCT04266301 STIMULUS-MDS2 (CMBG453B12301)
Indication	Myelodysplastic syndrome	Myelodysplastic syndrome
Phase	Phase 2	Phase 3
Patients	120	500
Primary Outcome Measures	Complete Remission (CR) rate and Progression Free Survival (PFS)	Overall survival
Arms/Intervention	• Experimental: Sabatolimab (MBG453) + hypomethylating agents • Placebo comparator: Placebo + hypomethylating agents	• Sabatolimab 800 mg + azacitidine 75 mg/m^2 • Sabatolimab 800 mg + azacitidine 75 mg/m^2 + placebo
Target Patients	Adult subjects with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as per IPSS-R criteria	Patients with intermediate, high or very high risk Myelodysplastic Syndrome (MDS) as Per IPSS-R, or Chronic Myelomonocytic Leukemia-2 (CMML-2)
Read-out Milestone(s)	H2-2021 (CR)	2023
Publication	Abstract submission to congress in H2-2021	TBD

MBG453 – TIM-3 antagonist

Study	NCT04150029 STIMULUS-AML1 (CMBG453C12201)
Indication	Acute Myeloid Leukemia (AML)
Phase	Phase 2
Patients	86
Primary Outcome Measures	• Incidence of dose limiting toxicities (Safety run-in patients only) • Percentage of subjects achieving complete remission (CR)
Arms/Intervention	• Single arm safety and efficacy study of sabatolimab in combination with azacitidine and venetoclax
Target Patients	Newly diagnosed adult AML patients who are not suitable for treatment with intensive chemotherapy
Read-out Milestone(s)	2024
Publication	TBD

PDR001 – PD-1 checkpoint inhibitor

Study	NCT03484923 (CPDR001J2201)
Indication	Previously treated unresectable or metastatic melanoma
Phase	Phase 2
Patients	195
Primary Outcome Measures	Objective Response Rate (ORR)
Arms/Intervention	• Spartalizumab (PDR001) 400mg i.v. Q4W + LAG525 (to be tested in unselected patients and LAG-3 positive patients) • Spartalizumab 400mg i.v. Q4W + capmatinib • Spartalizumab 400mg i.v. Q4W + canakinumab • Spartalizumab 400mg i.v. Q4W + ribociclib
Target Patients	Adult patients with previously treated unresectable or metastatic melanoma
Read-out Milestone(s)	2022
Publication	TBD

Promacta®/Revolade® – Thrombopoietin receptor agonist

Study	NCT03025698 (CETB115E2201)	NCT03988608 (CETB115E2202)
Indication	Previously untreated or relapsed/refractory severe aplastic anemia or recurrent aplastic anemia	Previously untreated or relapsed/refractory severe aplastic anemia or recurrent aplastic anemia
Phase	Phase 2	Phase 2
Patients	60	20
Primary Outcome Measures	PK of eltrombopag at steady state in pediatric patients with SAA	Hematologic response rate rate up to 26 weeks of treatment
Arms/Intervention	• Eltrombopag 12.5, 25, 50, 75 mg FCT & 25 mg pFOS • Arm A: relapsed/refractory SAA or recurrent AA following IST for SAA: hATG/cyclosporine + eltrombopag or cyclosporine + eltrombopag • Arm B: previously untreated SAA: hATG/cyclosporine + eltrombopag	• Eltrombopag 25 mg film-coated tablets
Target Patients	Pediatric patients from age 1 <18 years with relapsed/refractory SAA or recurrent AA after IST or previously untreated SAA	Chinese patients with refractory or relapsed severe aplastic anemia
Read-out Milestone(s)	Primary: 2022 Final: 2025	Primary: 2021 Final: 2023
Publication	TBD	TBD

Rydapt® – Multi-targeted kinase inhibitor

Study	NCT03280030 (CPKC412A2220)	NCT03591510 (CPKC412A2218)
Indication	Acute myeloid leukemia	Acute myeloid leukemia
Phase	Phase 2	Phase 2
Patients	66	50
Primary Outcome Measures	Incidence of safety events and event free survival	Occurrence of dose limiting toxicities Event Free Survival (EFS)
Arms/Intervention	• Midostaurin 50 mg • Placebo	• Chemotherapy followed by Midostaurin
Target Patients	Newly diagnosed patients with FLT3-mutated acute myeloid leukemia (AML) from pan-Asia countries	Newly diagnosed pediatric patients with FLT3 mutated acute myeloid leukemia (AML)
Read-out Milestone(s)	Interim: Q2-2020 *(actual)*; Final: H2-2021	2025
Publication	Abstract submission to congress in H2-2021	TBD

SEG101 – p-Selectin inhibitor

Study	NCT03264989 SOLACE-Adults (CSEG101A2202)	NCT03474965 SOLACE-Kids (CSEG101B2201)
Indication	Prevention of Vaso-Occlusive Crises (VOC) in patients with Sickle Cell Disease (SCD)	Prevention of VOC in pediatric patients with SCD
Phase	Phase 2	Phase 2
Patients	57	100
Primary Outcome Measures	PK/PD and safety of SEG101 (crizanlizumab) at 5 mg/kg	PK/PD and safety of SEG101 at 5 mg/kg
Arms/Intervention	SEG101 (crizanlizumab) at a dose of 5.0 mg/kg (or 7.5 mg/kg for exploratory group) by IV infusion, ± Hydroxyurea/Hydroxycarbamide	SEG101 (crizanlizumab) at a dose of 5 mg/kg by IV infusion ± Hydroxyurea/Hydroxycarbamide
Target Patients	Adult SCD patients with VOC	Pediatric SCD patients with VOC
Read-out Milestone(s)	2019 *(actual)*	H2-2021 (pediatric patients ≥12 year old) 2024 (pediatric patients <12 year old)
Publication	Liles D, et al. Presented at ASH 2020	Planned abstract submission to congress in H2-2021

SEG101 – p-Selectin inhibitor

Study	NCT03814746 STAND (CSEG101A2301)
Indication	Prevention of Vaso-Occlusive Crises (VOC) in patients with Sickle Cell Disease (SCD)
Phase	Phase 3
Patients	240
Primary Outcome Measures	Rate of VOC events leading to healthcare visit
Arms/Intervention	• Crizanlizumab 5.0 mg/kg • Crizanlizumab 7.5 mg/kg • Placebo
Target Patients	Adolescent and adult SCD patients (12 years and older)
Read-out Milestone(s)	2022
Publication	TBD

Tafinlar® – BRAF inhibitor

Study	NCT01677741 (CDRB436A2102)
Indication	BRAFV600 mutant cancers
Phase	Phase 1/2
Patients	85
Primary Outcome Measures	Safety, tolerability and pharmacokinetics
Arms/Intervention	Single-arm study of oral dabrafenib (dose based on age and weight)
Target Patients	Pediatric subjects aged 1 year to <18 years with advanced BRAF V600-mutation positive solid tumors
Read-out Milestone(s)	H1-2021
Publication	• Kieran MW et al. Clin Cancer Res 2019;25(24):7294-7302 (PK analysis) • Hargrave DR et al. Clin Cancer Res 2019;25(24):7303-7311 (safety/efficacy in low-grade gliomas)

Tafinlar®+Mekinist® – BRAF inhibitor and MEK inhibitor

Study	NCT02684058 (CDRB436G2201)
Indication	BRAFV600 mutant gliomas
Phase	Phase 2
Patients	142
Primary Outcome Measures	Objective response rate
Arms/Intervention	Dabrafenib + trametinib (dose based on age and weight)
Target Patients	Children and adolescent patients with BRAF V600 mutation positive relapsed or refractory high grade glioma (HGG) or BRAF V600 mutation positive low grade glioma (LGG)
Read-out Milestone(s)	2022
Publication	TBD

Tafinlar®+Mekinist® – BRAFV600 inhibitor and MEK inhibitor

Study	NCT02124772 (CTMT212X2101)
Indication	BRAFV600 mutant solid tumors
Phase	Phase 1/2A
Patients	139
Primary Outcome Measures	Safety, tolerability and pharmacokinetics and clinical activity
Arms/Intervention	Trametinib (dose based on age and weight) Dabrafenib + trametinib (dose based on age and weight)
Target Patients	Pediatric Subjects Aged 1 Month to <18 Years with Advanced V600-Mutation Positive Solid Tumors
Read-out Milestone(s)	H1-2021
Publication	• Geoerger B, et al. Presentation at ASCO 2020 • Manuscript submission Q4-2020

^{177}Lu-PSMA-617 – Radioligand therapy targeting prostate specific membrane antigen (PSMA)

Study	NCT03511664 VISION (PSMA-617-01)
Indication	PSMA-positive Metastatic Castration-resistant Prostate Cancer (mCRPC)
Phase	Phase 3
Patients	831
Primary Outcome Measures	• Radiographic Progression Free Survival • Overall Survival
Arms/Intervention	• 177Lu-PSMA-617 plus BS/BSC • BS/BSC alone
Target Patients	Adult patients with PSMA-positive Metastatic Castration-resistant Prostate Cancer (mCRPC)
Read-out Milestone(s)	H1-2021
Publication	H2-2021

^{177}Lu-PSMA-617 – Radioligand therapy targeting prostate specific membrane antigen (PSMA)

Study	NCT04720157 (CAAA617C12301)	NCT04689828 (CAAA617B12302)
Indication	metastatic Hormone Sensitive Prostate Cancer	pretaxane 2L mCRPC
Phase	Phase 3	Phase 3
Patients	~1126	~495
Primary Outcome Measures	Radiographic Progression Free Survival (rPFS)	Radiographic Progression Free Survival (rPFS)
Arms/Intervention	• Arm 1: 177Lu-PSMA-617 Participant will receive 7.4 GBq (+/- 10%) 177Lu-PSMA-617, once every 6 weeks (+/- 1 week) for a planned 6 cycles, in addition to the Standard of Care (SOC); ARDT +ADT is considered as SOC and treatment will be administered per the physician's order • Arm 2: For participants randomized to Standard of Care arm, ARDT +ADT is considered as SOC and treatment will be administered per the physician's order	• Arm 1: Participants will receive 7.4 GBq (200 mCi) +/- 10% 177Lu-PSMA-617 once every 6 weeks for 6 cycles. Best supportive care, including ADT may be used • Arm 2: For participants randomized to the ARDT arm, the change of ARDT treatment will be administered per the physician's orders. Best supportive care, including ADT may be used
Target Patients	Patients with metastatic Hormone Sensitive Prostate Cancer (mHSPC)	mCRPC patients that were previously treated with an alternate ARDT and not exposed to a taxane-containing regimen in the CRPC or mHSPC settings
Read-out Milestone(s)	Primary 2024	2024
Publication	TBD	TBD

Lutathera® – Radioligand therapy targeting somatostatin receptor type 2

Study	NCT03972488 NETTER-2 (CAAA601A22301)
Indication	Gastroenteropancreatic neuroendocrine tumors (GEP-NET)
Phase	Phase 3
Patients	222
Primary Outcome Measures	• Progression Free Survival
Arms/Intervention	• Lutathera plus long-acting octreotide • high dose long-acting octreotide
Target Patients	Adult patients with Grade 2 and Grade 3 Advanced GEP-NET
Read-out Milestone(s)	2023
Publication	TBD

TNO155 – SHP2 Inhibitor

Study	NCT03114319 (CTNO155X2101)	NCT04000529 (CTNO155B12101)
Indication	Solid tumors (single agent)	Solid tumors (combo)
Phase	Phase 1	Phase 1
Patients	255	~126
Primary Outcome Measures	• Number of participants with adverse events • Number of participants with dose limiting toxicities	• Incidence of dose limiting toxicities (DLTs) during the first cycle of combination treatment during the dose escalation part • Incidence and severity of adverse events (AEs) and serious adverse events (SAEs) as per CTCAE v5.0, by treatment • Dose tolerability
Arms/Intervention	• Drug: TNO155 • Drug: TNO155 in combination with EGF816 (nazartinib)	• TNO155 and Spartalizumab (PDR001) • TNO155 and Ribociclib (LEE011)
Target Patients	Adult patients with advanced solid tumors in selected indications	Patients with advanced malignancies.
Read-out Milestone(s)	2023	2022
Publication	TBD	TBD

NIS793 – TGFβ1 inhibitor

Study	NCT02947165 (CNIS793X2101)
Indication	Solid tumors
Phase	Phase 1
Patients	120
Primary Outcome Measures	• Incidence of DLTs, AEs, SAEs and dose reductions / interruptions for NIS793 • Incidence of DLTs, AEs, SAEs and dose reductions/interruptions for NIS793 in combination with PDR001
Arms/Intervention	• NIS793 • NIS793 + PDR001
Target Patients	Adult patients with advanced malignancies
Read-out Milestone(s)	2021
Publication	TBD

Ophthalmology

Lucentis® – Anti-VEGF

Study	NCT02640664 RAINBOW Extension (CRFB002H2301E1)
Indication	Retinopathy of Prematurity (ROP)
Phase	Phase 3
Patients	180
Primary Outcome Measures	To evaluate the visual function of patients by assessing the visual acuity in the better-seeing eye at the patient's fifth birthday.
Arms/Intervention	• Ranibizumab 0.2 mg (up to Week 40, if warranted) • Ranibizumab 0.1 mg (up to Week 40, if warranted)
Target Patients	Male and female preterm infants with bilateral retinopathy of prematurity (ROP) who completed RAINBOW.
Read-out Milestone(s)	2023
Publication	TBD

Beovu® – Anti-VEGF

Study	NCT04005352 TALON (CRTH258A2303)	NCT03710564 MERLIN (CRTH258AUS04)
Indication	Neovascular Age-related Macular Degeneration (nAMD)	Neovascular Age-related Macular Degeneration (nAMD)
Phase	Phase 3B	Phase 3
Patients	~692	~530
Primary Outcome Measures	• Average change in Best-corrected visual acuity • Distribution of the last interval with no disease activity (in a Treat-to-Control regimen)	Change from baseline in Best-Corrected Visual Acuity (BCVA)
Arms/Intervention	Arm 1: Brolucizumab 6 mg intravitreal injection Arm 2: Aflibercept 2 mg intravitreal injection	Arm 1: Brolucizumab 6 mg for intravitreal injection Arm 2: Aflibercept 2 mg for intravitreal injection
Target Patients	Patients with Neovascular Age-related Macular Degeneration (nAMD) who have not previously received anti-VEGF (vascular endothelial growth factor) treatment	Patients with Neovascular Age-related Macular Degeneration (nAMD) with persistent retinal fluid
Read-out Milestone(s)	2022	H1-2021
Publication	TBD	TBD

Beovu® – Anti-VEGF

Study	NCT03386474 (CRTH258A2301E1)	NCT03481634 KESTREL (CRTH258B2301)
Indication	Neovascular age-related macular degeneration (nAMD)	Diabetic eye disease
Phase	Phase 3	Phase 3
Patients	150	534
Primary Outcome Measures	Number of treatment-emergent adverse events	Change from baseline in best-corrected visual acuity (BCVA)
Arms/Intervention	• Brolucizumab (RTH258) 6 mg/50 µL • Aflibercept 2 mg/50 µL	• Brolucizumab (RTH258) 3 mg/50 µL • Brolucizumab (RTH258) 6 mg/50 µL • Aflibercept 2mg/50 uL
Target Patients	Patients with neovascular age-related macular degeneration who have completed the CRTH258A2301 study	Patients with visual impairment due to diabetic macular edema (DME)
Read-out Milestone(s)	2018 *(actual)*	Primary: Q4-2020; Final: H2-2021
Publication	Planned publication of the attributes of brolucizumab and durability in H1-2021	Week 52 safety and efficacy data of KITE and KESTREL studies combined in 1 abstract to be submitted to ARVO (May 2021) with additional submissions planned to ASRS, Euretina, AAO

Beovu® – Anti-VEGF

Study	NCT03481660 KITE (CRTH258B2302)	NCT04058067 KINGLET (CRTH258B2304)
Indication	Diabetic eye disease	Diabetic macular edema
Phase	Phase 3	Phase 3
Patients	356	268
Primary Outcome Measures	Change from baseline in best-corrected visual acuity (BCVA)	Change in best-corrected visual acuity (BCVA)
Arms/Intervention	• Brolucizumab (RTH258) 6 mg/50 µL • Aflibercept 2 mg/50 µL	• Brolucizumab (RTH258) 6 mg/50 µL • Aflibercept 2 mg/50 µL
Target Patients	Patients with visual impairment due to diabetic macular edema (DME)	Chinese patients with visual impairment due to diabetic macular edema
Read-out Milestone(s)	Primary: Q3-2020 *(actual)*; Final: H2-2021	2023
Publication	Week 52 safety and efficacy data of KITE and KESTREL studies combined in 1 abstract to be submitted to ARVO (May 2021) with additional submissions planned to ASRS, Euretina, AAO	Publication planned for 2023

Beovu® – Anti-VEGF

Study	NCT03917472 KINGFISHER (CRTH258B2305)	NCT03802630 RAPTOR (CRTH258C2301)
Indication	Diabetic macular edema	Retinal vein occlusion
Phase	Phase 3	Phase 3
Patients	500	500
Primary Outcome Measures	Change in best-corrected visual acuity (BCVA) from baseline up to week 52	Change from baseline in best-corrected visual acuity (BCVA) at week 24
Arms/Intervention	• Brolucizumab (RTH258) 6 mg/50 µL • Aflibercept 2 mg/50 µL	• Brolucizumab (RTH258) 6 mg/50 µL • Aflibercept 2 mg/50 µL
Target Patients	Patients with visual impairment due to diabetic macular edema	Adult patients with visual impairment due to macular edema secondary to branch retinal vein occlusion
Read-out Milestone(s)	H2-2021	2023
Publication	Publication submission planned for 2022	Publication submission planned for 2024

Beovu® – Anti-VEGF

Study	NCT03810313 RAVEN (CRTH258C2302)	NCT04047472 HOBBY (CRTH258A2307)
Indication	Retinal vein occlusion	Macular degeneration
Phase	Phase 3	Phase 3
Patients	750	494
Primary Outcome Measures	Change from baseline in best-corrected visual acuity (BCVA) at week 24	Change from baseline in best-corrected visual acuity (BCVA) at week 48
Arms/Intervention	• Brolucizumab (RTH258) 6 mg/50 µL • Aflibercept 2 mg/50 µL	• Brolucizumab (RTH258) 6 mg/50 µL • Aflibercept 2 mg/50 µL
Target Patients	Adult patients with visual impairment due to macular edema secondary to central retinal vein occlusion	Chinese patients with neovascular age-related macular degeneration
Read-out Milestone(s)	2023	2024
Publication	TBD	TBD

Beovu® – Anti-VEGF

Study	NCT04278417 (CRTH258D2301)
Indication	Diabetic retinopathy
Phase	Phase 3
Patients	706
Primary Outcome Measures	Change from Baseline in BCVA
Arms/Intervention	• Arm 1: RTH258 (Brolucizumab) 6 mg3 x q6w loading injections, followed by q12w maintenance through week 90 • Arm 2: Panretinal photocoagulation laser initial treatment in 1-4 sessions up to Week 12, followed with additional PRP treatment as needed
Target Patients	Patients with proliferative diabetic retinopathy
Read-out Milestone(s)	2023
Publication	TBD

ECF843A – Lubrification / anti-inflammatory

Study	NCT04391894 (CECF843A2201)
Indication	Dry Eye Disease
Phase	Phase 2
Patients	680
Primary Outcome Measures	• Change from baseline in symptom assessment in Dry Eye (SANDE) score • Change from baseline in composite corneal fluorescein staining score
Arms/Intervention	A Study to Assess the Safety and Efficacy of ECF843 vs Vehicle in Subjects with dry eye disease ECF843 0.15 or 0.45 mg/mL BID/TID/vehicle
Target Patients	Patients with moderate to severe dry eye disease (DED)
Read-out Milestone(s)	H2-2021
Publication	2022

Respiratory

INC424 – JAK Inhibitor

Study	NCT04362137 RUXCOVID (CINC424J12301)
Indication	COVID-19 (cytokine storm)
Phase	Phase 3
Patients	402
Primary Outcome Measures	Proportion of patients who die, develop respiratory failure (requires mechanical ventilation), or require intensive care unit care
Arms/Intervention	• Ruxolitinib 5 mg tablet given bid • Placebo
Target Patients	Patients with COVID-19 respiratory disease
Read-out Milestone(s)	Dec 2020 (Actual)
Publication	• Manuscript submission planned for Q1-2021

QBW251 – CFTR potentiator

Study	**NCT04072887 (CQBW251B2201)**
Indication	Chronic obstructive pulmonary disease (COPD)
Phase	Phase 2
Patients	956
Primary Outcome Measures	Trough FEV1 (Forced Expiratory Volume in 1 second) change from baseline after 12 weeks of treatment
Arms/Intervention	• QBW251 450 mg • QBW251 300 mg • QBW251 150 mg • QBW251 75 mg • QBW251 25 mg • Placebo
Target Patients	COPD patients on background triple inhaled therapy (LABA / LAMA / ICS)
Read-out Milestone(s)	H1-2022
Publication	Manuscript submission planned for 2022

CSJ117 – TSLP inhibitor

Study	NCT04410523 (CCSJ117A12201C)
Indication	Asthma
Phase	Phase 2
Patients	625
Primary Outcome Measures	Pre-dose FEV1 (Forced Expiratory Volume in 1 second) change from baseline after 12 weeks of treatment. Average change from baseline in pre-dose FEV1 at week 8 & week 12
Arms/Intervention	• CSJ117 0.5mg • CSJ117 1mg • CSJ117 2 mg • CSJ117 4 mg • CSJ117 8 mg • Placebo
Target Patients	Asthma patients on background medium or high ICS *plus* LABA therapy
Read-out Milestone(s)	2022
Publication	TBD

QVM149 – Long-acting beta2 agonist, Long-acting muscarinic antagonist and inhaled corticosteroid

Study	NCT03100500 (CQVM149B1305)	NCT03100825 (CQVM149B1304)
Indication	Asthma	Asthma
Phase	Phase 3	Phase 3
Patients	51	94
Primary Outcome Measures	Long-term safety/tolerability: Incidence and severity of treatment emergent adverse events during the 52 weeks study	Long-term safety/tolerability: Incidence and severity of treatment emergent adverse events during the 52 weeks study
Arms/Intervention	• Single arm: QMF149 150/320 µg od	• Single Arm: QVM149 150/50/160 µg od
Target Patients	Japanese patients with asthma inadequately controlled	Japanese patients with asthma inadequately controlled
Read-out Milestone(s)	2019 *(actual)*	2019 *(actual)*
Publication	• Sagara H, et al. Abstract presented at ATS 2020 • Planned publication in Q1-2021	• Nakamura Y, et al. Abstract presented at ATS 2020 • Planned publication in Q1-2021

Sandoz Biopharmaceuticals

Hyrimoz® – Biosimilar adalimumab

Study	NCT02744755 ADMYRA (GP17-302)
Indication	Immunology
Phase	Phase 3
Patients	353
Primary Outcome Measures	Change in DAS28-CRP score from baseline to week 12 in patients treated with GP2017 and patients treated with Humira®
Arms/Intervention	• GP2017 • US licensed Humira® adalimumab
Target Patients	Patients with moderate to severe active rheumatoid arthritis
Read-out Milestone(s)	2018 *(actual)*
Publication	• Wiland, P. et al., presented at EULAR 2019 • Wiland, P. et al., BioDrugs, Q2-2020

GP2411 – Biosimilar denosumab

Study	NCT03974100 (CGP24112301)
Indication	Osteoporosis
Phase	Phase 3
Patients	522
Primary Outcome Measures	Percent change from baseline (%CfB) in lumbar spine Bone Mineral Density
Arms/Intervention	• GP2411 60 mg /mL subcutaneous injection every 6 months • Prolia® 60 mg /mL subcutaneous injection every 6 months
Target Patients	Postmenopausal women with osteoporosis
Read-out Milestone(s)	2022
Publication	Study data publications expected for 2024 and beyond. The overall study design will be published at WCO and ECTS congresses 2020.

Global Health

COA566 – PGH-1

Study	NCT04300309 CALINA (CCOA566B2307)
Indication	Malaria, uncomplicated (<5kg patients)
Phase	Phase 3
Patients	44
Primary Outcome Measures	Artemether Cmax
Arms/Intervention	• Experimental: artemether lumefantrine (2.5 mg:30 mg) • artemether lumefantrine (2.5 mg:30 mg) bid over 3 days, from 1-4 tablets per dose
Target Patients	Infants and Neonates <5 kg body weight with acute uncomplicated plasmodium falciparum malaria
Read-out Milestone(s)	Primary outcome measure: 2023
Publication	• TBD

KAF156 – Plasmodium Falciparum Inhibitor – PfCARL mediated

Study	NCT03167242 (CKAF156A2202)	NCT04546633 KALUMI (CKAF156A2203)
Indication	Malaria	Malaria uncomplicated
Phase	Phase 2	Phase 2
Patients	~500	224
Primary Outcome Measures	PCR-corrected Adequate Clinical and Parasitological Response (ACPR)	PCR-corrected and uncorrected Adequate Clinical and Parasitological Response (ACPR)
Arms/Intervention	• KAF156 and LUM-SDF (different combinations) • Coartem	• KAF156 and LUM-SDF QD (once daily) for 2 days in fasted condition • KAF156 and LUM-SDF QD (once daily) for 2 days in fed condition
Target Patients	Adults and children with uncomplicated Plasmodium Falciparum Malaria	Malaria patients -< 18 years old with malaria caused by P. falciparum
Read-out Milestone(s)	H2-2021	2022
Publication	• Two posters accepted, ASTMH meeting Nov 15-19 2020 • Kublin JG et al. Clinical Infectious Diseases 09 Jul 2020, PMID: 32644127	TBD

Abbreviations

aBC	Advanced breast cancer
AD	Atopic Dermatitis
AIH	Autoimmune hepatitis
aHUS	atypical Hemolytic Uremic Syndrome
ALL	Acute lymphoblastic leukemia
ALS	Amyotrophic lateral sclerosis
AMI	Acute myocardial infarction
AML	Acute myeloid leukemia
AS H2H	Ankylosing spondylitis head-to-head study versus adalimumab
BC	Breast cancer
C3G	C3 glomerulopathy
CCF	Congestive cardiac failure
CLL	Chronic lymphocytic leukemia
CML	Chronic myeloid leukemia
CRC	Colorectal cancer
COPD	Chronic obstructive pulmonary disease
COSP	Chronic ocular surface pain
CRSwNP	Severe chronic rhinosinusitis with nasal polyps
CSU	Chronic spontaneous urticaria
CVRR-Lp(a)	Secondary prevention of cardiovascular events in patients with elevated levels of lipoprotein (a)
CVRR-LDLC	Secondary prevention of cardiovascular events in patients with elevated levels of LDLC
DME	Diabetic macular edema
DLBCL	Diffuse large B-cell lymphoma refractory
FL	Follicular lymphoma
GCA	Giant cell arteritis
GVHD	Graft-versus-host disease
HCC	Hepatocellular carcinoma
HD	Huntington's disease
HFpEF	Chronic heart failure with preserved ejection fraction
HF-rEF	Chronic heart failure with reduced ejection fraction
HNSCC	Head and neck squamous cell carcinoma
HS	Hidradenitis suppurativa
IA	Interim analysis
IgAN	IgA nephropathy
iMN	Membranous nephropathy
IPF	Idiopathic pulmonary fibrosis
JIA	Juvenile idiopathic arthritis
LVEF	Left ventricular ejection fraction
mCRPC	Metastatic castration-resistant prostate cancer
MDR	Multi-drug resistant
MDS	Myelodysplastic syndrome
MS	Multiple sclerosis
wAMD	Wet (neovascular) age-related macular degeneration
NASH	Non-alcoholic steatohepatitis
nHCM	Non-obstructive hypertrophic cardiomyopathy
nr-axSpA	Non-radiographic axial spondyloarthritis
NSCLC	Non-small cell lung cancer
PDR	Proliferative diabetic retinopathy
PEF	Preserved ejection fraction
PedPsO	Pediatric psoriasis
PNH	Paroxysmal nocturnal haemoglobinuria
PsA	Psoriatic arthritis
RCC	Renal cell carcinoma
PROS	PIK3CA related overgrowth spectrum
RA	Rheumatoid arthritis
rMS	Relapsing multiple sclerosis
ROP	Retinopathy of prematurity
RP	Retinitis pigmentosa
RVO	Retinal vein occlusion
SAA	Severe aplastic anemia
SLE	Systemic lupus erythematosus
SMA Type 1	Spinal muscular atrophy (IV formulation)
SMA Type 2/3	Spinal muscular atrophy (IT formulation)
SpA	Spondyloarthritis
SPMS	Secondary progressive multiple sclerosis
TNBC	Triple negative breast cancer
T1DM	Type 1 Diabetes mellitus